

National Assembly for Wales

Medicines funding in the NHS October 2011

Issues around the availability of medicines and the perceived 'postcode lottery' in the NHS have been widely reported in the media.

This paper examines how medicines come to be approved for use within the NHS in Wales, and the routes available for patients/clinicians to access medicines that are not routinely funded.

It includes information on policy elsewhere in the UK, such as the Cancer Drugs Fund in England. It also outlines the UK Government's proposals to reform the pricing system for medicines.

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Medicines funding in the NHS

1. Introduction

Cases of patients being denied access to certain drugs on the NHS are often highlighted in the media. Assembly Members may also be aware of constituents who have faced difficulty accessing what they, and their doctor, feel is the most appropriate treatment for them. The apparent inequalities in the availability of medicines across Wales and the UK have been the subject of widespread debate and comment in the UK legislatures and among stakeholder organisations.

At present, before new medicines can be routinely used to treat NHS patients, they undergo an appraisal process to determine whether the benefit to patients justifies the cost. Following appraisal, a minority of new medicines are not deemed clinically and cost effective enough to be recommended for general use within the NHS.

Ultimately, however, it is a clinician's decision as to which drug is the best treatment for their patient and clinicians can choose to prescribe any medicine - including unlicensed medicines - if their Local Health Board, or individual patient, is willing to pay for it.

Lack of a consistent approach across Health Boards to handling requests for those medicines not routinely available has been blamed for fuelling the so-called 'postcode lottery' of NHS drugs funding.

This research paper explores the process of assessment of new medicines for use within the NHS, and what this means for patients wanting to access medicines that are not recommended for general use.

This paper also outlines the changes proposed by the UK Government to the pricing system for medicines. It also sets out the current appraisal process in Scotland for comparison.

2. Licensing of medicines

A manufacturer can only market a medicine once it has received a licence, known as a marketing authorisation. Only medicines which meet the required standards of safety, quality and efficacy are granted a marketing authorisation.

This licence describes the condition(s) the medicine can be used to treat, the way in which it can be given and the dosage, and which patients can be treated.

The licensing process is not influenced by the cost effectiveness or value for money of a medicine; these issues are considered by the relevant UK bodies who decide which treatments to recommend to their respective health systems (see next section - *The appraisal process* for further information on this). Nor does it restrict the prescribing of any medicine by a doctor, provided the doctor has assured themselves that the medicine is safe and effective for that patient.

In the European Union (EU), there are two ways of obtaining a marketing authorisation for a medicine:

- the centralised procedure, via the European Medicines Agency, which results in a single marketing authorisation valid throughout the EU;
- national authorisation procedures, where individual EU Member States authorise medicines for use in their own territory.

All human medicines intended for the treatment of HIV/AIDS, cancer, diabetes, neurodegenerative diseases, auto-immune and other immune dysfunctions and viral diseases must be approved via the European Medicine Agency's centralised procedure. The same applies to officially-designated 'orphan medicines' (used for the treatment of rare human diseases) as well as to all advanced-therapy medicines (such as gene-therapy) and medicines derived from biotechnology processes.

Each EU Member State has its own procedures for the authorisation, within their own territory, of medicines that fall outside the scope of the centralised procedure. The relevant national authority for human medicine in the UK is the Medicines and Healthcare products Regulatory Agency (MHRA).

The European Medicines Agency and MHRA in the UK are also responsible for supervising the safety of medicines after they have been authorised.

3. The appraisal process

The National Institute for Health and Clinical Excellence (NICE) provides advice to the NHS on both the clinical effectiveness and cost effectiveness of a selected group of newly-licensed medicines. This 'technology appraisal', as it is known, is a significant part of NICE's current role. NICE's programme of appraisals is set by the Department of Health in consultation with the Welsh Government.

NICE's advice has a statutory basis in England and Wales, and Local Health Boards (LHBs) are legally obliged to fund medicines approved by NICE within three months of publication of the advice.

Possible changes to NICE's role are looked at in section 7 - *Pricing of medicines and the future role of NICE*.

In 2000, the then Minister for Health and Social Services, Jane Hutt AM, established a Task and Finish Group for Prescribing, to identify ways to improve the availability of new medicines in Wales. An All Wales Medicines Strategy Group (AWMSG) was set up in 2002 to advise on the introduction of medicines (particularly high-cost medicines) at a national level.

The New Medicines Group (NMG) is a sub-group of the All Wales Medicines Strategy Group. NMG make preliminary recommendations to the AWMSG in relation to each new medicine undergoing appraisal. The Welsh Medicines Partnership (WMP) provides the secretariat to AWMSG.

Since April 2009, in order to put AWMSG guidance on an equal statutory footing with NICE guidance in Wales, there has been a legal requirement for Local Health Boards to fund medicines recommended by AWMSG within three months of endorsement by the Minister for Health and Social Services.

Which medicines are appraised?

Initially, AWMSG was tasked with the appraisal of the clinical and cost effectiveness of high-cost medicines which were not on the NICE work programme¹.

From April 2007, AWMSG broadened its appraisal process to include all new cancer and cardiac therapies in addition to the high-cost medicines (up to a maximum of 32 medicines per year).

In December 2009, the then Minister for Health and Social Services, Edwina Hart AM, agreed that, from October 2010, the remit of AWMSG would be further broadened to appraise **all** new medicines not on the NICE work programme (up to

¹ A high-cost medicine was defined as one likely to cost £2,000 per patient per year or greater.

70 additional appraisals per year). To avoid duplication of effort, AWMSG would not normally undertake an appraisal if NICE intend to publish final advice on the same product within 12 months. (Should NICE subsequently publish guidance on the same product, this supersedes AWMSG's advice).

Specialist groups such as the All Wales Cancer Drugs Group (AWCDG) and the New Cardiovascular Drugs Group (NCDG) may provide advice on new medicines within their areas, helping to inform AWMSG's appraisal programme.

4. Exceptional funding

If a particular medicine has not been recommended by NICE/AWMSG for general use within the NHS in Wales, a clinician can make an individual application to an LHB for exceptional funding for the medicine on behalf of their patient. This is also commonly referred to as 'individual patient funding' or 'individual patient treatment'.

A 2008 survey by Macmillan Cancer Support² highlighted a wide variation in exceptional funding processes across Wales.

The 2009 'Routledge Report' – *Towards improving the availability of medicines for patients in Wales*³, commissioned by the Welsh Government, recommended that a national guideline be developed to ensure 'robust, consistent, transparent, inclusive and timely consideration of requests for exceptional funding of medicines'. An Implementation Group was established to take the Routledge Report's recommendations forward. The Group published their report⁴ in June 2010, which stated that an all Wales approach to managing requests for individual patient funding was being developed by the Welsh Government.

In April 2011, the Rarer Cancers Foundation charity published a report - *Exceptional Cymru? - An audit of the progress made in improving access to treatment for people with rarer cancers in Wales*⁵. The audit was undertaken using information provided by LHBs following requests from the Rarer Cancers Foundation made under the *Freedom of Information Act 2000*. The Foundation's report stated that a significant number of Welsh patients are denied access to cancer treatment and that variations remain across Wales. It made a number of recommendations for the Welsh Government, including the provision of updated guidance to Local Health Boards on handling applications for exceptional funding and developing a nationwide process for determining applications to fund off-label treatment (further information on off-label treatment is given below).

In answer to a Written Assembly Question on 7 June 2011, the Minister for Health and Social Services, Lesley Griffiths AM, stated:

To ensure funding decisions are fair and the definition of exceptionality is consistently applied across NHS Wales, an All-Wales Policy for Making Decisions on Individual Patient Requests has been developed. The policy is in its final draft and will be finalised very shortly. However all NHS organisations are already working to the principles of the policy, which will

² Macmillan Cancer Support, *Cancer patients facing exceptional difficulties to get funding for drugs*, October 2011 [accessed 07 September 2011]

³ All Wales Medicines Strategy Group, *Towards improving the availability of medicines for patients in Wales - Expert Group Review*, January 2009 [accessed 1 September 2011]

⁴ Implementation Group, *Improving the availability of medicines for patients in Wales - Implementation Group Report*, March 2010 [accessed 1 June 2011]

⁵ Rarer Cancers Foundation, *Exceptional Cymru? An audit of the progress made in improving access to treatment for people with rarer cancers in Wales*, April 2011 [accessed 1 September 2011]. The report forms part of the Rarer Cancers Foundation's public policy work, which receives some financial support from pharmaceutical companies.

ensure transparency, equity and accountability. The new arrangements will ensure that patients and their referring clinicians are fully engaged and communicated with throughout the process.⁶

In July 2011, the Welsh Government published the all Wales policy – *Making Decisions on Individual Patient Funding Requests (IPFR)*⁷. The policy sets out the principles underpinning how decisions to approve or decline such requests are made, and the process for making these decisions.

Off-label and unlicensed treatment

Clinicians have the flexibility to prescribe medicines outside of their licensed indication where they judge this to be in the best interest of the patient on the basis of available evidence. This is known as ‘off-label’ use. Such practice is common in paediatrics for example, where difficulties in the development of age-appropriate formulations mean that many medicines prescribed for children are used off-label. Off-label treatment may also be an issue for patients with cancer, particularly rarer cancers. For example, where there is no licensed medicine for a particular type of rare cancer, a clinician may wish to prescribe a medicine licensed for a more common cancer which has a similar underlying disease process (this type of treatment may also be referred to as ‘near-label’).

Off-label treatment falls outside the remit of AWMSC and NICE appraisals, and so would usually only be provided on the NHS through an individual patient funding request.

As indicated earlier in this briefing, clinicians are also able to prescribe unlicensed medicines where they consider there to be no alternative, licensed medicine that would meet the patient's needs. NHS funding for unlicensed medicines would also be subject to an individual patient funding request.

When prescribing unlicensed or off-label treatment, full clinical and professional responsibility rests with the prescribing clinician.

⁶ WAQ57426, [Darren Millar to Lesley Griffiths \(Minister for Health and Social Services\)](#), 7 June 2011 [accessed 1 September 2011]

⁷ Welsh Government, [All Wales Policy – Making Decisions on Individual Patient Funding Requests \(IPFR\)](#), July 2011 [accessed 6 September 2011]

5. Top-up payments

If NHS funding is not granted, and a clinician wishes to prescribe an unfunded medicine, a patient may decide to purchase the treatment themselves. This has been commonly referred to as ‘top-up payments’ (the terms ‘co-payments’, ‘patient contributions’ and ‘user charges’ are also used).

The issue of top-up payments was addressed in a 2008 report for the UK Secretary of State for Health, the ‘Richards Report’⁸. The Report makes a number of recommendations which are intended to set out a clear framework for how the NHS in England should handle situations where patients might wish to purchase additional drugs, but also to keep to a minimum the number of patients who will be placed in this position by ensuring that as many clinically effective drugs as possible are provided on the NHS. The Report recommended that if patients do opt to purchase additional medicines, they should not lose entitlement to NHS care that they would otherwise have received.

The Implementation Group on improving the availability of medicines for patients in Wales, referred to in the previous section, considered and endorsed the recommendations of the Richards Report but identified a lack of clarity on implementation in Wales with regard to the issue of top-up payments. Successful implementation of some of the Routledge Report’s other recommendations (timely appraisals of new medicines by AWMSG and developing a consistent approach to handling requests for exceptional funding for example) were recognised as key to minimising the need for top-up payments.

In July 2010, the then Minister for Health and Social Services, Edwina Hart, invited the Implementation Group to consider the legal and ethical framework around top-up payments, and to develop clarification on the procedures for patients wishing to fund private packages of care. This work was undertaken against a background that “NHS Wales would maintain its focus on ensuring timely, appropriate, evidence based access to cost effective medicines for all”, and that “the key principles of the NHS will be retained regardless of whether or not top-ups are introduced.”⁹

The Group’s report on top-up payments was published in February 2011. The report states:

When an individual funding request has failed on grounds of cost effectiveness it is proposed that as long as the medicine can be shown to have a favourable benefit to harm profile for that individual patient, private payment may be permitted to allow an individual to

⁸ Richards, M., *Improving access to medicines for NHS patients: a report for the Secretary of State for Health by Professor Mike Richards*, November 2008 [accessed 30 August 2011]

⁹ Implementation Group, *Improving the availability of medicines for patients in Wales – Top-up Payments - Implementation Group Report*, February 2011 [accessed 5 October 2011]

access the medicine. This may supplement or replace other medicines the patient is receiving from the NHS for the treatment of their particular condition. The top-up treatment package will be delivered as a separate, discrete episode of care but the patient will continue to be an NHS patient for all other ongoing episodes of care.¹⁰

In March 2011, LHBs were advised to adopt the recommendations in the Group's report.¹¹

¹⁰ *ibid*

¹¹ All Wales Medicines Strategy Group, *Prescribing Dilemmas: A Guide for Prescribers*, August 2011 [accessed 5 October 2011]

6. Cancer Drugs Fund in England

On 1 April 2011, following consultation¹², the UK Government launched a £200 million a year 'Cancer Drugs Fund'. The Fund is intended to provide a means of improving patient access to cancer drugs in England prior to an anticipated reform of pricing arrangements for branded medicines.

The Secretary of State for Health, Andrew Lansley MP, said:

My priority is to give everyone, including cancer patients, better access to drugs and innovative treatments on the NHS.

We will do this by reforming the way drug companies are paid for NHS medicines. As an interim measure, we are creating a new Cancer Drug Fund, which will operate from April 2011. This fund will help patients get the drugs their doctors recommend.¹³

Further information on the UK Government's proposals to reform medicines pricing is given in the following section – *Pricing of medicines and the future role of NICE*.

The Cancer Drugs Fund will operate for three years from 1 April 2011. It follows interim cancer drugs funding of £50m which began on 1 October 2010.

The Fund is intended to complement, not replace, existing funding processes and Primary Care Trusts in England are expected to continue to consider requests for exceptional funding. The Fund is coordinated on a regional basis by Strategic Health Authorities (SHAs).

The First Minister answered the following Assembly Question on 1 March 2011:

Nick Bourne: ... I have a specific question about the cancer drugs fund that will be available in England and which will be well-funded. We would introduce such a fund in Wales. Cancer is an issue of massive concern throughout the United Kingdom. What will you do to deal with the dreadful scourge of cancer, and, in particular, to deal with the availability of cancer drugs?

The First Minister: Our record on cancer is a good one. We note what has happened in England with the cancer drugs fund, but we do not see the need for a separate cancer drugs fund in Wales. All new drugs that are deemed clinically cost-effective by the National Institute for Health and Clinical Excellence, or the all-Wales medicine strategy group, are provided on the NHS. In Wales, we spend £19.70 more per head of population on treating cancer than is spent in England. It may well be that the cancer drugs fund is needed in England to make up a shortfall that does not exist in Wales.¹⁴

¹² Department of Health, *The Cancer Drugs Fund - A consultation*, October 2010 [accessed 18 August 2011]

¹³ Department of Health press release, *Response to plans by Asda to sell some cancer drugs at cost price*, 21 May 2010 [accessed 30 August 2011]

¹⁴ *RoP p4*, 1 March 2011 [accessed 18 August 2011]

The Rarer Cancer Foundation's report of April 2011, referred to earlier, highlights cross-border inequalities and states:

Currently there are 22 treatments available in England which are not routinely available in Wales on the grounds that they have not been recommended by NICE or the AWMSG. Clinicians in England are able to apply for these treatments through the Cancer Drugs Fund whereas clinicians in Wales have no other option than to pursue funding through the exceptional case process.¹⁵

¹⁵ Rarer Cancers Foundation, *Exceptional Cymru? An audit of the progress made in improving access to treatment for people with rarer cancers in Wales*, April 2011[accessed 7 September 2011].

7. Pricing of medicines and the future role of NICE

This section is concerned with the pricing of **branded** medicines – medicines with a proprietary name – which account for around 80 per cent of the NHS drugs bill.

(Medicines marketed without a brand name are known as generic medicines. They account for approximately four fifths of prescriptions by volume, but only around one fifth of the overall cost of medicines. The reimbursement costs for generic medicines in England and Wales are set out in the Department of Health's Drug Tariff¹⁶.)

The Pharmaceutical Price Regulation Scheme (PPRS) is the method by which the Department of Health (on behalf of the UK health departments) seeks to control the prices of branded medicines.

PPRS has existed since 1957 and is usually renegotiated every five years. It is a voluntary scheme agreed between the Department of Health and the Association of the British Pharmaceutical Industry (ABPI).

The Scheme has sought to achieve a balance between reasonable prices for the NHS and a fair return for the industry to enable it to research, develop and market new and improved medicines. Under PPRS, pharmaceutical companies have freedom of pricing for new active substances. However, PPRS controls the prices of branded medicines through regulating the profits that pharmaceutical companies are allowed to make on their sales to the NHS.

Earlier this year, the Department of Health consulted on proposals for a new value-based system of pricing medicines¹⁷. The stated aim of value-based pricing is to improve NHS patients' access to effective and innovative drugs by ensuring they are available at a price that reflects the value they bring, as assessed on the basis of improvements in health outcomes. The UK Government intends to move to this system when the current Pharmaceutical Price Regulation Scheme (PPRS) expires at the end of 2013. The consultation closed on 17 March 2011.

The proposals had implications for NICE's role in the appraisal of new medicines. The Department of Health website states, in respect of policy in England:

Much of the kind of work NICE currently does will still be needed to support the process of determining a value-based price. And clinicians will still need authoritative advice on how new and existing treatments best fit into the treatment for a particular condition. That is why we are confident that, while NICE's role will inevitably change under new a [sic] value-based pricing system, it will continue to have an important part to play.¹⁸

¹⁶ NHS Business Services Authority, *National Health Service England and Wales Electronic Drug Tariff* [accessed 7 September 2011]

¹⁷ Department of Health, *A new value-based approach to the pricing of branded medicines - A consultation*, December 2010 [accessed 1 September 2011]

¹⁸ *NICE: frequently asked questions*, Department of Health [accessed 1 September 2011]

The proposals in England have raised questions about the future role of NICE in Wales. The First Minister responded to the following Assembly Question in Plenary on 2 November 2010:

Helen Mary Jones: First Minister, do you agree that one potentially serious threat to the health budget in Wales and over the border - although, of course, that is not a matter for us - is the decision that the Westminster Government has taken effectively to deregulate medicines? It has decided to remove from the National Institute for Health and Clinical Excellence its right to determine value for money, and that could potentially lead to a complete free-for-all. While it might have some benefits for some individual patients, it will overall benefit the wealthy pharmaceutical companies much more. Will you undertake to discuss with the Minister for Health and Social Services how we can ensure that whatever is done to deregulate the provision of medicines in England, we are not forced down the same path here in Wales?

The First Minister: We were not aware of what was happening with NICE, even though it is an England-and-Wales body, and that raises several issues. First, the idea behind NICE was that it would approve drugs that, in a way, went beyond the marketing of those drugs by the drug companies. As someone who knows many GPs, I know full well that the marketing was pretty generous to GPs at one time. The idea behind NICE was to take that element away. The difficulty for GPs in England now is that they will not be able to refer to an independent body that has approved a particular drug or not, and they will get the blame if they are not able to prescribe certain drugs. I do not think that GPs in Wales would welcome being put in that position.¹⁹

The UK Government published their response to the consultation on 18 July 2011. In response to concerns over changes to NICE's role, this stated:

We have listened to the concerns raised about the need for a mechanism to support patient access to drugs with a value-based price. As set out above [in the response], through a funding direction, we currently require NHS commissioners in England to fund drugs and treatments in line with NICE's recommendations. We can confirm our intention, set out in the Government response to the Future Forum report, to maintain the effect of the funding direction in the value-based pricing arrangements, to ensure that the NHS in England consistently funds medicines with a value-based price. The NHS will be required to fund drugs already recommended by NICE, as well as drug treatments subject to the value-based pricing regime. This means patients will continue to have the legal right to clinically appropriate, cost-effective drugs and treatments as set out in the NHS Constitution and accompanying handbook.²⁰

In respect of how a value-based pricing policy may apply across the UK, the response stated:

It is important that there is a common medicines pricing policy across the UK and, like the PPRS, we expect value-based pricing to be a UK-wide system. However, the Devolved

¹⁹ [RoP p7](#), 2 November 2010 [accessed 18 August 2011]

²⁰ UK Government, [A new value-based approach to the pricing of branded medicines - Government response to consultation](#), 18 July 2011 [accessed 6 September 2011]

Administrations determine many aspects of health policies, including those affecting the use and availability of medicines within their health systems. We will work closely with our counterparts in the health departments of the Devolved Administrations to ensure a coherent approach.²¹

²¹ *ibid*

8. Assessment of new medicines in Scotland

In Scotland, the organisations responsible for providing guidance to the NHS on the use of new medicines are the Scottish Medicines Consortium (SMC) and Healthcare Improvement Scotland²².

SMC, under the umbrella of Healthcare Improvement Scotland, advises on the clinical effectiveness and cost effectiveness of all newly licensed medicines.

Medicines that SMC recommends for use for conditions where alternative drug treatments already exist are subject to local NHS Board decisions; an NHS Board will review a particular medicine in the context of other existing comparable medicines on its approved list (known as a 'local formulary'). This means that, although treatment for a clinical condition is available uniformly across Scotland, there may be variation in the individual drugs chosen locally to treat that clinical condition.

SMC may designate an innovative medicine for a condition where there are no other treatment options as 'unique'. If such a medicine for a specific condition was recommended by SMC, NHS Boards would be required to introduce it in an agreed national programme.

Medicines not recommended by SMC for use within the NHS in Scotland may be made available in certain circumstances through individual patient treatment (exceptional funding) requests.

Healthcare Improvement Scotland also considers NICE multiple technology appraisal (MTA) guidance²³, and will advise NHS Boards on whether this guidance should be applicable in Scotland. If Healthcare Improvement Scotland validates a NICE MTA recommendation, NHS Boards in Scotland are required to make the recommended medicine(s) available.

²² Healthcare Improvement Scotland is a health body established on 1 April 2011. It will build on work previously undertaken by NHS Quality Improvement Scotland and the Care Commission.

²³ NICE's MTA process is designed to appraise single or multiple products, devices or other technologies, with one or more indications. The 'single technology appraisal' (STA) process is specifically designed for the appraisal of a single product, device or other technology, with a single indication.