

The teeth

in the NHS Constitution:

the case of the right to NICE approved treatments

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National Voices

People shaping health
and social care



Preface

This discussion paper examines whether patients are achieving one of the most identifiable of their healthcare rights – the right to receive treatments approved by NICE (the National Institute for Health and Clinical Excellence) for use in the NHS in England.

In the face of persistent reports that this right was being breached, National Voices wanted to know whether the following questions could be answered:

- Are patients getting the treatments they are entitled to?
- If not, why not?
- And if not, what can be done to improve their access?

Although the right to NICE-approved treatments appears to be straightforward, and has been approached by successive governments as such, this inquiry was anything but straightforward.

Put simply, data to assess whether patients are achieving their right, or whether there are significant groups who are being prevented from getting access, is simply not available to a high enough standard.

This begs further questions. If we cannot monitor the impact and utility of this simplest of rights, then what is the meaning of a patient ‘right’ in the NHS?

How effective can the NHS Constitution, which codifies our rights, hope to be? Is there anything that can be done to find teeth for it? And how should patient groups and organisations respond?

We do not pretend to be experts in this area. But we do represent patients’ interests – National Voices is a coalition of over 130 patient and service user charities – so if we cannot divine what is going on, then there is a problem.

In the context of the NHS drive for ‘innovation’, and the government’s continuing work on strengthening the NHS Constitution, we offer our findings and reflections for debate.

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Executive summary

This discussion paper examines whether patients in England are achieving a specific healthcare right, to receive treatments approved by NICE.

This right is in the NHS Constitution, and is based on directions from the Secretary of State under the NHS Act 2006. At time of publication it relates to 275 health technologies appraised by NICE since March 2000.

The government has been active since 2011 to reinforce implementation of this right, through top-down initiatives under the 'Innovation, Health and Wealth' banner, including a compliance regime; an implementation push; and a public innovation 'scorecard'.

However, we simply do not know whether patients are achieving their right, or the extent of any barriers in their way. There is no adequate data to tell us.

National Voices sought to add to what is known through literature searches and a comprehensive survey of local/regional and national patient groups in England.

Some 665 patient groups completed at least part of the survey. The most common conditions focused on by these groups were cancer, neurological conditions and rare diseases.

Many respondents believed there were problems with access to treatments, with only 15% of national and 21% of regional groups saying access was 'excellent' or 'good'. Asked for their direct knowledge of people being unable to access NICE-approved treatments they reported a minimum of 4,928 affected patients. They also identified a range of minority groups who they believed suffered greater inequality of access.

Among the reasons they cited for problems with access were:

- differing interpretations of NICE guidance by PCTs
- differing interpretations of NICE guidance by clinicians
- bureaucratic delays
- prescriber policy of favouring cheaper alternatives
- the fact that the therapy is unconventional
- clinicians unaware of NICE guidance
- lack of infrastructure/resources at local level and
- lack of staff.

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Patient groups had tried a variety of routes to pressure the system into compliance – going via the Department of Health, Strategic Health Authorities, Primary Care Trusts, individual clinicians and NHS providers. They reported great variability in the willingness of these organisations to respond.

However, we treat all the survey responses with caution, because the other factor the survey revealed was widespread confusion about this topic among patient groups. For example, many were not aware of what Health Technology Appraisals (HTAs) are, or did not discriminate between them and other ‘guidance’ issued by NICE, such as clinical guidelines (which are non-mandatory).

Some reported ‘lack of access’ precisely because there were no treatments for their conditions, approved or otherwise. Others may have been concerned with different treatment funding decisions taken legitimately at local level for non-appraised treatments.

Our literature searches indicated that, based on sales and other data, a positive appraisal from NICE is likely to trigger increased usage of a treatment. However, a negative appraisal does not necessarily lead to lower usage.

We found examples of positive HTAs having only a partial effect on local clinical practice. Clinicians appear to resist some new treatments; or to implement them partially; or to continue to use alternatives that they prefer. There is clearly a problem translating mandatory guidance to the clinical level.

Among the reasons suggested for this were:

- *timing* of the appraisal publication, and the context into which it is released
- *systemic weaknesses* in some NHS organisations that prevent or delay the implementation, such as failure to put in place proactive processes to anticipate and absorb new guidance; lack of awareness and dissemination of recommendations through organisations; inability rapidly to provide the necessary staff, facilities or equipment for implementation; failure to audit, review and change practice at the clinical level, and to tackle areas of non-compliance
- *funding* – where providers and their clinicians often have a perception that funding has not been agreed; or that funding only covers the treatment, not related costs such as follow-up
- *attachment to local clinical practice* and/or ‘second-guessing’ of NICE guidance.

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We found no reliable studies of primary care trust policy and practice in this field, which is a major gap in knowledge. However, a case study from National Voices member the Royal National Institute of Blind People, focused on Ozurdex, found that 42% of NHS trusts it surveyed seven months after the positive HTA had a sub-standard service, and 12% had none at all. In follow-up, the RNIB was able to trace some of the reasons back to PCTs, for example:

- delaying the decision to implement
- restricting the number of those who could access the treatment
- withholding funding so that individual clinicians had to make individual applications
- having too many decision making bodies locally, and
- communicating poorly with provider trusts.

Taken together these findings suggest that:

- while there is no evidence to suggest that denial of this patient right is widespread, it does happen locally; and
- that the barriers to access are less to do with commissioner Primary Care Trust (PCT) funding policies and ‘second-guessing’ (though this exists) – but more to do with **poor NHS organisation, communication failures, and a failure to ensure that clinicians are informed, audited and supervised for compliance.**

These barriers cannot be addressed by further reinforcement of ‘top-down’ demands. Nor can they be addressed by ‘patient demand’, given the extent of confusion among patients and their groups about the guidance and their right.

Our investigation has significant implications for the NHS Constitution, which may be relevant to other ‘rights’ in the document.

This very clear and specific legal right is undermined because:

- *patients themselves are unaware of the right*, uncertain about what it means in practice, and easily confused by the overlap with other, non-rights-based aspects of access to treatment;
- *the NHS has not organised itself specifically to address the monitoring of the right*, starting with the absence of data to assess its achievement. For all rights in the Constitution, an evidence base is essential, as is an active approach by NHS England to monitor achievement;

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- *there is no single, direct route to redress.* This is common to all the Constitutional rights – various overlapping mechanisms exist for patients to raise concerns, and they cannot be clearly advised which to use.

In the ‘suggested actions’ section National Voices offers a number of ideas for addressing these deficits, as follows:

1. We suggest that NICE, with the Secretary of State’s approval, should re-categorise its HTAs to reflect their non-negotiable status – for example, as ‘technology directions’.

If NICE HTAs are mandatory by law, they are not ‘guidance’ – they are ‘requirements’. A change in terms may help to reduce confusion with the many other forms of ‘guidance’ issued by NICE.

2. We suggest that the Secretary of State, NHS England and others should consider the case for requiring that compliance with each technology appraisal should be independently audited within, say, 15 months of publication (that is, one year after the deadline for implementation).

We further suggest that provisions to require all clinicians to participate in these and in national clinical audits should be strengthened throughout the NHS in England.

Clinical audit is the single most effective way to tackle the barriers we identified at the level of local clinical practice, clinician lack of awareness and/or resistance, and providers’ failure to ensure compliance.

Systematic audit would also serve up better data about the actual (rather than ‘expected’) use of approved treatments.

3. Data could be improved nationally and locally, by involving patient organisations:

- a) to conduct national surveys of patients and of NHS organisations to establish the true prevalence of need, and the actual usage of approved treatments; and
- b) to co-produce local data for public health and joint strategic needs assessments.

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4. There should be a single route to redress for any patient experiencing barriers to accessing approved treatments.

We suggest this should be by appeal to the Clinical Commissioning Group (CCG) for a review, with a time limit for their response.

5. NHS England's process for authorising CCGs should include an element of assessing their adherence to GPG1 (the NICE guidance on best practice in formularies).

NHS England more generally needs to demonstrate, early on, that it recognises compliance with HTAs as a priority for the new system, and is making plans to inform, guide and monitor CCGs with regard to implementation.

6. The Secretary of State and NHS England should consider how to standardise the workings of formularies in local areas, including whether there can be a 'single formulary' approach in each locality.

7. The government, NHS England and the NHS Constitution Expert Working Group should balance continued use of top-down 'compliance approaches' with more research and investigation into local realities and population needs.

8. Public Health England, with Directors of Public Health and key voluntary organisations, should consider how, over time, public health and needs assessment data can be systematically improved and developed to the point where it might act as baseline data of actual need for HTA-approved treatments.

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The right to NICE-approved treatments

The NHS Constitution (Department of Health, 2012a)¹ states, as a patient ‘right’:
“You have the right to drugs and treatments that have been recommended by NICE for use in the NHS, if your doctor says they are clinically appropriate for you.”

The Constitution, by agreement of its original drafters, does not create legal rights, it only codifies and groups them. The rights must already exist in legislation.

According to the Constitution Handbook the source of this right is found in directions from the Secretary of State, as follows:

“Directions given by the Secretary of State to Primary Care Trusts on the funding of guidance in National Institute for Health and Clinical Excellence (NICE) technology appraisals. The directions are made under section 8 of the NHS Act 2006 and primary care trusts have a legal obligation to comply with such Directions.

“The Directions require Primary Care Trusts to apply funding so as to ensure that a treatment covered by an appraisal is normally available within three months after the date of publication of the appraisal.”

The Health and Social Care Act 2012 maintains the relevant regulations and directions. However, since April 2013 the directions apply to the Clinical Commissioning Groups (CCGs) that have replaced Primary Care Trusts.

How NICE approves treatments

The reference in the Constitution to ‘drugs and treatments that have been recommended by NICE’ means those which have been the subject of positive Health Technology Appraisals carried out by NICE.

NICE assesses not just medicines but also medical devices, diagnostic techniques (tests to identify diseases), surgical procedures, and health promotion activities.

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NICE examines the clinical evidence for the treatment, and its value for money.

The outcome of the appraisal can either be:

- a positive recommendation for use in the NHS, or
- an 'optimised' recommendation
 - usually specifying its use for a smaller sub-set of patients than originally intended, or
- for use only in clinical research trials, or
- not recommended.

There is a continuing question over the adequacy of the data used to assess clinical effectiveness. Pharmaceutical companies do not publish all the data they own about clinical trials, and critics argue that all of this should be available to NICE in making its judgements.

Most HTAs result in either a positive or an optimised recommendation – that is, NICE rarely says an outright 'no' to a new medicine or treatment. This should not be surprising, bearing in mind that the medicine or treatment in question will have already been through an evidenced process of being authorised for licence in the UK market – that is, its makers will have marshalled evidence from trials that it can be effective.

Between March 2000 and March 2013 NICE published 275 Health Technology Appraisals.

It is important to note that Health Technology Appraisals are termed 'guidance' by NICE, and form **one part** of a suite of guidance documents for the NHS.

Thus the term '[NICE guidance](#)' refers not only to HTAs but also to:

- clinical guidelines
- Quality Standards
- interventional procedures guidelines
- public health guidance
- medical technologies guidance
- diagnostics guidance.

NICE is also assuming responsibility for guidance on social care.

The problem of variation

Many decisions on whether to fund drugs and treatments in the English NHS are made locally, historically at the level of Primary Care Trusts or Strategic Health Authorities (SHAs) and now by clinical commissioners. This creates the strong probability that some treatments will be available in one part of the country but not in another.

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This kind of variation offends against the popular and political belief that the NHS must provide fairly and equitably – an ‘NHS for all’ no matter who you are or where you live.

There are two kinds of variation – ‘warranted’ and ‘unwarranted’. A ‘warranted’ variation would be where an area has a high proportion of people with a particular disease or condition, and therefore makes a ‘reasonable’ decision to fund more of the appropriate treatments.

An ‘unwarranted’ variation is where treatment levels vary significantly with no obvious cause or reasonable justification.

Unwarranted variation is a widespread problem in the NHS in England. The NHS itself now publishes an annual Atlas of Variation, designed to make such variations transparent and thereby to enable local NHS organisations to recognise and tackle them.²

According to the NICE website,³ the explicit aim of Health Technology Appraisal is to reduce variation:

“NICE is asked to look at particular drugs and devices when the availability of the drug or device varies across the country. This may be because of different local prescribing or funding policies, or because there is confusion or uncertainty over its value. Our advice ends the uncertainty and helps to standardise access to healthcare across the country.”

However, over the last decade there have been complaints from patients, patient organisations and the media that, in fact, variation can persist long after a positive recommendation from NICE.

Indeed, some believe it can *result from* a positive NICE recommendation. That is because, it is alleged, while some parts of the country implement the recommendation immediately, other areas do not.

These accusations of geographical inequality are often termed ‘postcode lotteries’.

Patients’ decisions could be another source of ‘warranted’ variation. It is mandatory for the NHS to make available treatments approved by NICE, but of course it is not mandatory for the relevant patients to use them. Patients have other distinct rights in the NHS Constitution – to have information about *all* treatment options and their risks and benefits, and to be involved in their healthcare decisions. The principle of ‘shared decision making’ is that patients choose and agree with their clinicians which treatment is most appropriate for them.

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Variation – the need for caution

The subject of this paper is whether there are unwarranted variations in the provision of *NICE-approved* treatments.

However, three areas of confusion may creep into such discussions.

First, it is often hard to distinguish, especially in some media stories, between this – a variation in what is legally required to be available – and variation with regard to other drugs and treatments which have *not* been through the NICE HTA process, and where local funding decisions are therefore permissible (indeed necessary).

For example, in 2011 the government created a special Cancer Drugs Fund, which was allocated to the English NHS regions, so that patients could get additional access to new and innovative cancer treatments which may either be too expensive to achieve a NICE recommendation, or may take too long to go through that process.

Over the last two years, to judge by Google searching, the majority of media stories about variations in access to treatment have been about the use of these discretionary funds.⁴

Second, there is potential for confusion with regard to the different *types* of guidance provided by NICE, especially its clinical guidelines. These are longer and more complex documents that usually advise clinicians and NHS trusts on the best evidence-based practice for treating or managing specific conditions.

A guideline may cover several stages and treatment choices in a patient journey along a care ‘pathway’. These may or may not include the optional use of a treatment that has been recommended following an HTA. NICE guidelines are *not* legally mandatory for NHS organisations to implement.

Third, many of the actors involved in stories about ‘postcode lotteries’ may themselves be confused about the technical distinctions involved in this subject area, and so may complain that they are denied a ‘NICE-approved’ treatment when it features only in a guideline, and not in an HTA.

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Implementation

Until March 2013 the responsibility to implement a NICE HTA recommendation lay with Primary Care Trusts (PCTs).

PCTs maintained local 'formularies' – lists of drugs that are locally approved for use in the NHS. Clinicians are generally required to prescribe from this list. Where they wish to make a clinical case to prescribe a treatment that is not on the list, they must usually apply through a local 'appeal' mechanism, often known as an 'exemptions committee'.

Many of the accusations about patients being 'denied' drugs and treatments relate to PCT decisions on whether to fund them locally – that is, whether to include them in the formulary and thereby permit local clinicians to prescribe them.

NHS provider trusts may also hold formularies, which may affect how quickly NICE HTAs are locally implemented.

It is worth noting, however, for later discussion, that placing a drug in the formulary is not the *only* necessary step towards implementation. Clinicians and NHS providers have an important role to play – they decide, with their patients, what treatment options to take up.

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In December 2011 after a consultation, the Department of Health published ‘Innovation, Health and Wealth’, intended as an integrated set of measures to increase the scale and pace of innovation and diffusion across the NHS.⁵

The report had more significance than many of the DH’s general publications. It was healthcare’s response to an overall government drive for innovation, and simultaneously to the challenge of making the NHS more productive and sustainable. It bore a foreword from Sir David Nicholson, the then chief executive of the NHS who was also becoming the chief executive of the new NHS England. NHS England will take responsibility for making progress against its recommendations.

The report identified eight key themes, of which the first was: “We should reduce variation in the NHS and drive greater compliance with NICE guidance.”

Although this covers all NICE guidance — clinical guidelines as well as Health Technology Appraisals — the focus of the calls for action was on the latter. The report emphasised again that ‘there should be no legal barriers to accessing technologies recommended in NICE appraisals’ and that all such recommendations should be incorporated into local formularies within three months.

During 2012 the government followed up the report by developing three complementary policy activities: a compliance regime; an implementation push; and a public innovation ‘scorecard’.

The NICE compliance regime

In August 2012 Sir David Nicholson wrote to all NHS organisations to instruct them on compliance:

*“ [Formularies] should not duplicate NICE assessments or challenge an appraisal recommendation. Once on formularies, there should be no further barriers to the use or prescription of technologies or medicines.”*⁶

Therefore he wanted to see all NHS organisations publish information about which NICE

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Health Technology Appraisals were in their local formularies by 1st April 2013. Thereafter, it would be a requirement of their standard NHS contract to maintain the information, which must be ‘online... clear, simple and transparent’.

There was a further follow-up. The Chief Pharmaceutical Officer wrote again to these organisations later the same month informing them that he was heading a working group to ensure compliance with the regime, that NICE would issue good practice guidance on formularies by the end of 2012, and that he would ‘strongly encourage’ them to review their formularies to be compliant.

The NICE good practice guidance was issued in December 2012.⁷ Its recommendations emphasise that local formulary decision groups should:

- prioritise speed and efficiency, including continuous horizon scanning for new treatments that need consideration;
- work together so that all formularies in an area are making harmonious decisions;
- engage with stakeholders including patient and public representatives;
- discuss NICE HTAs at every meeting (and where the HTA is negative discuss withdrawing the treatment);
- automatically accept a treatment with a positive HTA, *and* incorporate it into care pathways, within three months.

The NICE Implementation Collaborative

‘Innovation, Health and Wealth’ promised to create a body including NHS organisations, NICE, industry bodies and professional leaders to support the implementation of NICE guidance.

This may include expanding NICE’s ability to provide ‘disinvestment’ advice, since one criticism of the NICE regime is that it recommends new treatments that must be funded, but does not guide the NHS on where to find the money by phasing out less effective interventions.

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The Innovation Scorecard

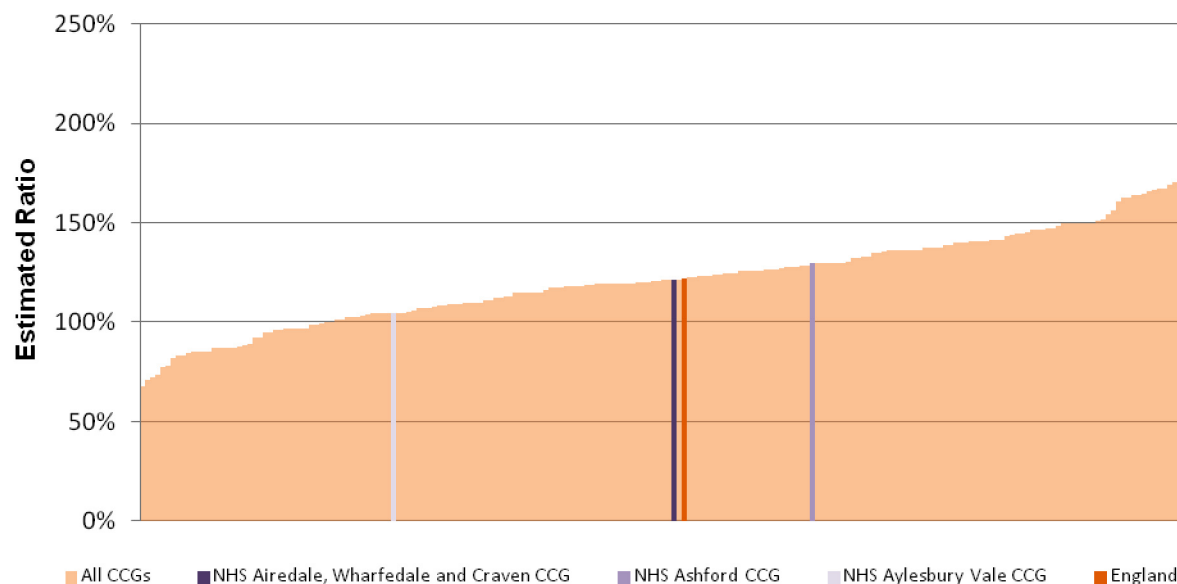
In January 2013 the NHS Information Centre (NHSIC) published the first ‘innovation scorecard’.⁸ Its declared aim is to publish transparent information on how well each NHS organisation is implementing NICE guidance.

This publication built upon a series of three previous attempts to capture what was happening nationwide regarding the use of approved treatments.⁹

The key difference this time was that the [report](#) was accompanied by an ‘interactive scorecard’.

This scorecard illustrates the variation from area to area in the use of NICE-approved medicines. These are shown, for example, in the form of charts such as the following:

Chart 1: ratio of observed and expected use of statins for all selected CCG areas in England



This shows how CCG areas to the left are making less use than expected of statins, while those to the right are making more use of them than expected.

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There is a series of tables, for all the treatments where it was possible to find data to compare observed and expected use, that show these kinds of variations broken down by strategic health authority area, by CCG area, and by NHS provider trust.

As the NHSIC notes in the full report:

“ This initial Innovation Scorecard is intended as an indicative measure in order to stimulate the monitoring of NHS compliance with NICETAs and of assisting the NHS in the identification of variation which, through discussion and commentary, can be explained, challenged or acted upon.”

That is, the hope is that by making explicit the variation between areas (or trusts), open publication of the data will provoke decision makers in those areas to review their practice with a view to reducing variation.

Various stakeholders had seen in this new scorecard a potential answer to the central problem of this discussion paper – how we can know whether this patient right is being achieved.

However, as the Association of British Pharmaceutical Industries (APBI), which strongly supported its development, has noted:¹⁰

“ the publication is experimental in nature and many more new medicines need to be included in the Scorecard in future if it is to become a useful, long-term tool... This first Scorecard is less detailed than the ABPI hoped for...”

The reason for this disappointment lies in the nature of the data.

As the title of the NHSIC report, and all previous publications in the series, make clear, these are ‘experimental statistics’ which come with significant caveats. The main problem is that they compare ‘observed’ (actual) with ‘expected’ uptake of treatments. As we discuss in the next section, this is a far from satisfactory measure.

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What do we already know? The data problem

When the NHS Future Forum, on behalf of the Secretary of State, began its review of the NHS Constitution in spring 2012, it was supported by Department of Health civil servants who were called on to provide evidence of the document's impact.

For evidence of whether patients were achieving their right to access NICE-approved treatments, they had only one set of data to rely on, compiled by the NHS Information Centre (NHS IC). An update (the third in this series) was later published in October 2012.¹¹ In January 2013 as described above, a new publication using data from 2011 was produced, together with the first 'innovation scorecard'.

All of these publications are described as 'experimental statistics'. This means, in the words of the NHS IC that they are "not fully developed and do not yet meet the quality standards of National Statistics". In other words, they should not be relied upon, and the NHS IC is at pains to remind readers of the reports – and users of the 'scorecard' – always to bear in mind the caveats and limitations that accompany them.

The base data used for these publications are drawn from the recording of prescribing decisions in primary and secondary care, as well as sales and purchasing information from industry and the NHS. These data enable a calculation of the actual, or 'observed' use of recommended treatments.

However, in order to know whether this uptake of a medicine reflects clinical *need*, we must be able somehow to predict how many people would use the medicine if the NICE recommendation was fully implemented.

Unfortunately there is no centrally collected data on how many patients have specific conditions that might require them to use relevant NICE-appraised treatments.

Thus in order to construct some kind of measurement of predicted usage the NHS IC combines three factors:

- an *estimate* of the number of eligible patients,
- an *assumed* figure for the *average* dose, and
- an *assumed* figure for the *average* length of treatment.

These three variables are all notional estimates, and that creates problems.

Note for transparency purposes: Sanofi, sponsor of this discussion paper, has membership of the two working groups overseeing this data

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Case studies discussed with patient organisations point to the fact that the number of people with their condition in the local population is often underestimated in official data. That may be exacerbated where people who are more likely to be excluded from mainstream services form a significant part of the population.

Where medicines are relatively new in the system, as is the case with many that go through HTAs, the likely dose and length of treatment are often uncertain, based largely on predictions from manufacturers' trial data, which may turn out not to be representative of how the medicine impacts on and is used by patients and their clinicians across a population.

The difficulties do not end there. For many of the HTA-approved medicines, the calculation of the predicted use proves impossible – as was the case for 12 of the 25 'groups' of treatments studied in 2010-11.

So just 13 groups of medicines were compared through this unsatisfactory method for 2010-11. And for the 'scorecard', just 19 medicines, relating to 13 HTAs, were included.

What, then, can we make of the calculations on these?

The 2010-11 report demonstrated variation by treatment. The ratio of observed versus predicted use varied markedly, from a drug that has only one tenth of the 'predicted' uptake, to one that appears to be more than 100% overprescribed. That excludes one medicine, Ranibizumab (trade name Lucentis), that appears three times overprescribed if the amount is measured in doses, but significantly underused if the amount is measured in vials.

However, the NHS IC itself cautions about interpreting the figures with such terms:

*“ it is important to note that predicted and observed use may differ for a variety of reasons and **they should not be assumed to definitely indicate either 'under' or 'over' prescribing.**” [our emphasis]*

In fact, it is not possible to deduce the reasons for these variations.

The 'scorecard' illustrates variation geographically. The data was drawn from the then existing PCT areas. But PCTs are being replaced by CCGs covering different territories

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from April 2013. So the NHS IC had to add another 'estimate' – the proportion of each PCT population that would now be covered by each CCG, based on Department of Health information.

The NHS IC is at pains to note that none of the data sets from which it has drawn for the 'scorecard' are related to clinical need, and that all have limitations or criticisms if they are to be used for assessing 'compliance'. Hence the report is clear that it cannot be used for performance management or for benchmarking.

Efforts by the Department of Health, industry and the NHS IC to improve these data analyses will continue, and the innovation scorecard is expected to become a regular publication. The NHS IC invites stakeholders to contribute to its considerations of better ways to develop the experimental statistics.

Nevertheless, from these efforts over the last three years one thing is clear – there is no reliable measure of compliance with or variation in the implementation of NICE technology appraisals.

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National Voices' research

Development

National Voices wanted to know to what extent English patients may be suffering inequalities in access to NICE-approved treatments.

Our principle line of enquiry would be through a survey of patient organisations, based on the reasoning that, if official data was inadequate, perhaps patients themselves could tell us what was happening.

There are many potential inequalities including:

- **global** — do English patients have better or worse access than people in other developed countries?
- **inter-UK** — do English patients have better or worse access than people in Scotland, Wales and Northern Ireland?
- **regional/local** — do people in some parts of England have worse access than in others?
- **personal** — do some people, by virtue of who they are, have worse access?
- **patient group** — do people with some diseases or conditions get worse access than others?

From an initial sounding with its members, National Voices concluded that the most common concern was regional/local variation.

At around the same time the government initiated a review of the NHS Constitution by the NHS Future Forum. This would involve assessing the impact of the Constitution on the NHS and its patients. The statement of each patient's right to access drugs approved by NICE, where clinically recommended, was among the most specific and identifiable of the patient entitlements encoded in the document.

Bearing these factors in mind we decided to focus our enquiry on examining the extent to which the right was being achieved. We then proceeded using the methods described below.

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Purpose and aims

The purpose of this project was to investigate the state of knowledge about inequalities in access to NICE-approved therapies, as reported by patient groups and evidenced in literature.

The aims were:

- to assess the extent to which patient groups knew about and were capable of reporting on such inequalities,
- to consider the future role of patients in monitoring and reporting on the achievement of the Constitutional right of access, and
- to consider and make recommendations to the NHS Future Forum, NICE and others with regard to future monitoring of this right.

Methods

The following research methods were used:

- a comprehensive survey of national and regional/local patient groups and organisations in England, and
- two literature searches.

The survey

To conduct the patient group survey we contracted [Patient View](#), a not-for-profit organisation with a comprehensive database of patient groups and organisations in the UK.

An online questionnaire was constructed with 12 main questions relating to the research topic.

We anticipated that regional/local groups might have a distinctly different knowledge and information base than national organisations, and therefore constructed two versions of the questionnaire.

Prior to the main topic questions, respondents were asked to answer six items relating to their own group/organisational profile and activities. The second of these asked whether the group covered all of England, or a part of it. Based on the response, the respondent was then routed either to the regional or national version of the questionnaire.

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The survey was distributed during June and July 2012 as a link within a covering email, through the following means:

- National Voices' member organisations via newsletters
- groups and organisations on the Patient View database
- the Patients Involved in NICE network of organisations
- other organisations via National Voices' external newsletters and other means.

Copies of the survey and the covering email are available on request from National Voices.

Patient View literature search

Patient View were also contracted to produce a literature search during July 2012. This was constructed as a wide-scale search, since we did not want to exclude any studies or papers that may be relevant.

As such the search terms covered 'NICE guidance' generally. We also looked for all forms of literature, including not just scholarly articles but also official reports and newspaper articles.

King's Fund literature search

Once we had absorbed and analysed the results of the first literature search we decided to conduct a second search to focus down specifically on Health Technology Appraisals, which were now used as the main search term. Again, we included all forms of literature. We contracted the library service at the [King's Fund](#) who carried out the search in October 2012.

Funding arrangement

The research and discussion paper were funded by an educational grant from [Sanofi](#), a multinational pharmaceutical company headquartered in Paris, which produces vaccines and treatments for use with diabetes, cancer and rare conditions. The funding

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agreement was made in line with the [National Voices ethical funding policy](#), which maintains the independence of the organisation's work and products with a framework of good governance.

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Findings (1): group patient survey

In this section we give the results of the patient survey.

The respondents

Respondents to national survey

Some 205 patient groups and organisations looked at the questionnaire and filled in at least one answer, and 92 completed it. This is a 'drop-off rate' of 55% (groups who started but did not complete the survey).

The majority of those who looked at the survey were groups whose focus included neurological conditions, cancer, rare diseases, and/or support to carers, family and friends (respondents were permitted to indicate more than one area of focus).

Of those who gave information, 14% of the groups focused solely on children, 9% solely on older people, and 7% on people from ethnic minorities.

A majority of respondents were engaged in the activities of providing information and support to patients, campaigning (including to raise awareness) and providing telephone helplines. Two fifths were involved in advocacy or representation to government and healthcare providers.

Respondents to regional/local survey

Some 460 patient groups looked at the questionnaire and filled in at least one answer, and 237 completed it: a drop-off rate of 49%.

The groups indicated they were evenly spread across most of the ten NHS regions in England, (8-13% each for seven of the regions); but only 5% came from the North East, while 17% and 20% respectively came from South West and South East England (including London).

The focus of these local respondents was slightly different to the national level. Among the areas of focus indicated by more than ten per cent of respondents, cancer,

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neurological conditions and support to carers, family and friends again featured, but the latter was the largest category. Unlike the national respondents, palliative care and mental health were also popular areas of focus.

A similar proportion of regional groups as national focused solely on older people (20%), but fewer on children (7% compared to 14%), and more on people from ethnic minorities (20%).

As with the national respondents, a large majority were engaged in providing information and support to patients, but only two fifths were involved in campaigning (including to raise awareness) and telephone helplines. They were much less likely to be involved in advocacy to healthcare providers (22%) or to government (11%).

Views about inequalities of access

Ratings of the quality of access

Respondents to the national survey were asked to comment on the quality of access to NICE-approved therapies ‘nationally, across England, for the kind of patients of whom you have knowledge’.

Of 106 national groups who answered this question, only 15% said access is either ‘excellent’ or ‘very good’. The biggest proportion, 46%, said it was ‘good, but patchy’, 18% said ‘poor’, while one fifth did not know.

The equivalent local question was for ‘your part of the country’. Here responses were more evenly spread, with 21% of the 252 respondents saying ‘excellent’ or ‘very good’; 18% ‘good’; and 27% ‘fair’ or ‘poor’. It was notable that some 35% said they did not know.

Discussion of the respondents

The fact that cancer, neurological conditions and rare diseases feature among the most frequently cited areas of focus is notable. These are the groups for whom access to medicines has been a burning issue; and where patient organisations have been involved in researching and campaigning on the barriers to access. Other types of patient groups may be less engaged or knowledgeable in this topic area.

The drop-off rate is very high for both versions of the questionnaire. This may be an indication that trigger words in the invitation to participate (‘inequalities’, ‘access’, ‘treatments’) motivated groups to embark on the questionnaire who then began to realise that they did not have specific knowledge of this topic.

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Numbers of people affected by problems with access

National groups were asked to estimate how many of the patients with whom they had contact had said they were not able to access NICE-approved treatments, even though they believed their medical circumstances justified such access.

Again, many of those looking at the questionnaire struggled to answer: 42% of the 106 respondents 'did not know'.

Another 21% were either unaware of any such patients, or worked in areas for which there was no NICE-approved treatment.

However, 39 national patient groups reported being directly aware of at least some patients for whom such access had been a problem, including three groups who said they were aware of more than 200 cases.

Altogether these 39 groups reported an incidence of at least 3,812 patients who had experienced problems with access.

Regional and local groups had less knowledge of such instances, with 69% unable to answer the question. Of the other 76 respondents, most reported low numbers of instances: 48 cited ten patients or less.

Nevertheless, altogether these groups were directly aware of a minimum of 1,116 cases of patients reportedly being unable to access NICE-approved treatments.

Adding the national to the regionally reported instances, that is a minimum of 4,928 affected patients.

Discussion

Caution should be exercised. First, these figures could potentially contain some double reporting. For example, a local group may refer a case to a national umbrella – 22 national organisations said they had received some cases from 'local members', though these could equally be individual as group members. Second, despite the specificity of the question, it remains possible/likely that some groups are reporting barriers to access that do not relate to treatments approved via a NICE Health Technology Appraisal.

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Inequalities by personal characteristics

We asked whether there were particular types of people who suffered from lack of access to NICE-approved therapies.

Among the national respondents there was a view that inequality was spread across population groups. Invited to tick more than one option, between nine and 15% of the national respondents to this question cited older people, people with learning disabilities, people with communication disabilities, the economically vulnerable, young people, people without English language, children, ethnic minorities and people with multiple chronic conditions.

Regional and local groups were more likely to indicate that older people, younger people or people with communication disabilities were experiencing inequality.

Cause of lack of access

We asked respondents to indicate the top three reasons they thought people were failing to get access to approved therapies.

Of the 69 national respondents to this question, 19 or more cited:

- differing interpretations of NICE guidance by PCTs
- differing interpretations of NICE guidance by clinicians
- bureaucratic delays
- prescriber policy of favouring cheaper alternatives
- the fact that the therapy is unconventional, and
- clinicians unaware of NICE guidelines.

The regional/local groups who responded cited the same factors but also cited:

- lack of infrastructure/resources at local level and
- lack of staff.

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Redress

Advocacy by national organisations

Some 51 national organisations reported that they had taken action to try to improve access to NICE-approved therapies.

We asked these respondents at which level they had challenged prescribing policy and/or clinical practice. The 30 national organisations that responded had mainly targeted PCTs, NHS providers and the Department of Health; but also cited specific consultants, specific GPs or GP practices and Strategic Health Authorities.

We then asked how willing these targets were to respond to the patient organisations' advocacy.

The specific consultants, NHS providers and Department of Health were all seen as 'very willing' or 'willing' by 40% or more of the respondents to this question. The managers of the NHS, however, were seen as much less willing to respond. None of the patient organisations saw either SHAs or PCTs as 'very willing'. PCTs were seen as 'willing' to respond by only ten per cent of the organisations that answered – and seen as 'unwilling' by 50%.

Advocacy by regional/local groups

Some 53 regional/local groups reported that they had taken action to try to improve access to NICE-approved therapies.

They were less likely to have targeted national institutions such as the Department of Health; and most respondents had focused on PCTs, NHS providers and specific consultants.

Notably, 16 of the regional/local groups said they had also contacted their Local Involvement Network (LINK).

Overall these regional/local respondents, like their national counterparts, also found NHS providers and specific consultants to be willing to respond to their advocacy.

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However, in sharp contrast to the national responses, over 60% of the regional/local respondents found that PCTs were either ‘very willing’ or ‘willing’ to respond.

They also reported LINKs as highly responsive with just under 70% saying they were ‘very willing’ or ‘willing’ to respond.

Using the NHS Constitution

We asked patient groups whether they had attempted to use the NHS Constitution to persuade NHS doctors or organisations to tackle access to NICE-approved therapies.

Only a small number had done so (fewer than ten national and fewer than 20 regional groups). Of these, even smaller numbers reported any degree of success in doing so (no more than seven national and eight regional/local groups).

Looking to the future

Finally we asked how the introduction of local clinical commissioning would affect access.

Of the 92 national organisations who answered the question, by far the largest category, 50%, expected the picture to vary across the country.

Regional/local organisations were asked to give a double response – for England as a whole and for their region or locality.

Of the small number who responded to the first part (23 groups), half expected the picture across England to vary, and another 30% thought access would reduce. For their own locality, around a third of the 38 organisations who responded said access would remain about the same, with another quarter saying it would get worse.

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Confusion: a further finding from the survey responses

National Voices wanted to know whether by asking patient groups and organisations across England, we could add to the picture available to judge whether patients are achieving their right to access NICE-approved drugs and treatments.

The email disseminating the online survey link to patient groups clearly specified the nature of the subject:

Dear [name of organisation]

National Voices is seeking your views on the subject of patient access to treatments that have been reviewed and approved by NICE.

Despite this clarity, the responses received indicated various degrees of confusion, misunderstanding or lack of knowledge among the groups who responded.

We concluded that this difficulty in distinguishing access to recommended treatments from other types of access or treatment issues was in itself a significant finding from the survey, and we discuss it below.

Significant lack of patient group knowledge and understanding

At least as significant as the informed responses we received were the uninformed responses.

There was considerable enthusiasm to contribute to a project about equality of access to treatment – some 665 patient groups attempted to respond. But large numbers of organisations did not complete the questionnaires, with drop-off rates of 55% for the national organisations and 49% for the regional and local groups.

This appears to indicate that the groups – many of which are engaged in advocacy for better treatment – did not have an informed understanding of what was, in fact, a very specific topic area.

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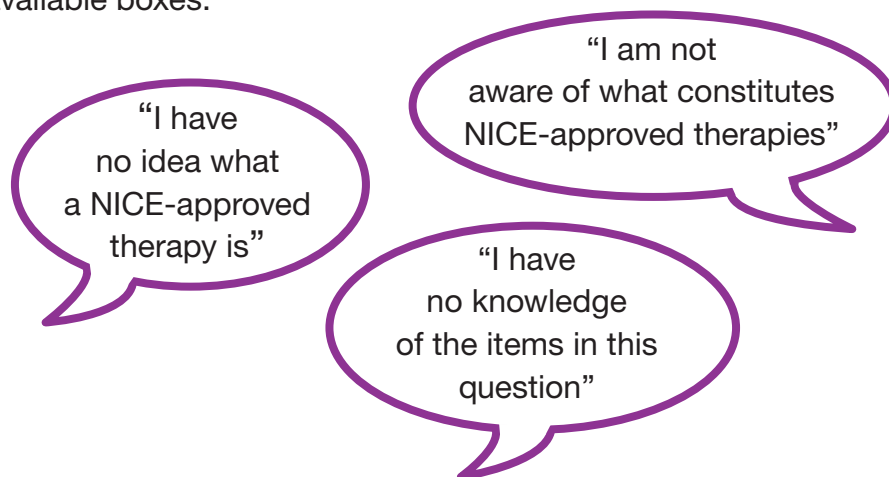
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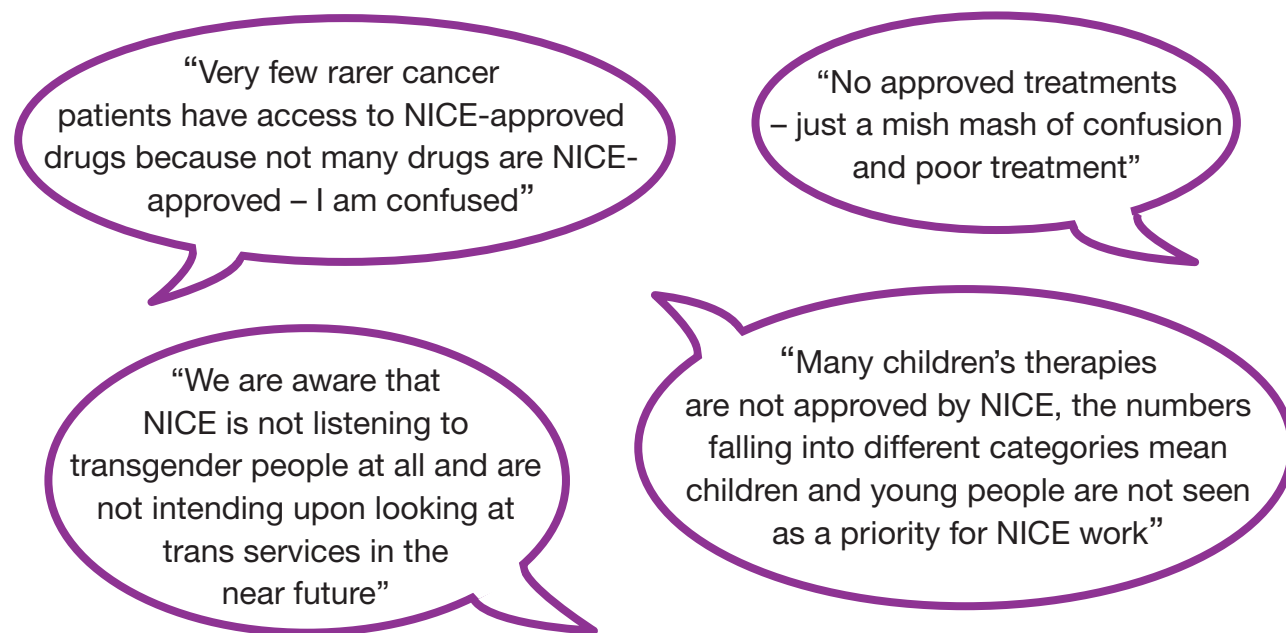
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That suspicion is reinforced by examining the free comments that respondents entered into the available boxes:



Some groups, especially those for less common conditions, were motivated to respond precisely by the absence of any NICE guidance in their area of concern. That is, their concern about lack of access was about the lack of any relevant technology appraisal recommendations – not the denial of access to those recommended treatments:



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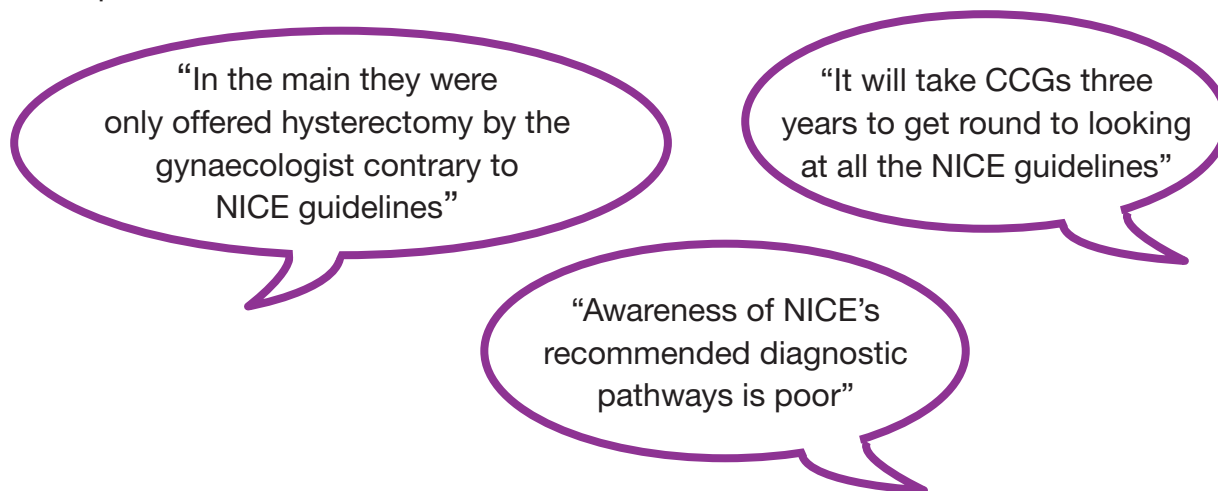
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Other groups had clearly understood the terminology of the survey as relating to any and all NICE guidance with which they might be concerned. They entered comments about diagnostic pathways, clinical guidelines and other aspects of guidance that were beyond the topic area:



Finally, a significant number of responding organisations were focused on cancer. They tend to have a heightened sensitivity to issues around denial of treatments. Online searches for ‘postcode lottery drugs’ produce a preponderance of cancer-related stories. However, these very often relate to the variability of PCT funding decisions about drugs that are not, or not yet, appraised by NICE (see, for example, ‘Child cancer drug postcode lottery revealed’, BBC News, 12 November 2012, a story resulting from TV journalism).

Again, it appears that these patient groups were motivated to start the questionnaire by their general concern about access to treatments, without having detailed knowledge or information about the specific topic.

Viewed online at
[www.bbc.co.uk/news/
uk-england-london-20272912#TWEET354252](http://www.bbc.co.uk/news/uk-england-london-20272912#TWEET354252)
12 November 2012

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Findings (2): literature reviews

National Voices commissioned two literature reviews for this discussion paper. The first, conducted by Patient View, was a wide ranging search for all materials about lack of adherence to NICE guidance. On examination, the results provided a wealth of material across a wide spread of disease areas; but many of the sources related to NICE guidance generally.

We therefore commissioned the King's Fund library service to conduct a second review, focused more specifically on NICE Health Technology Appraisals.

These were clearly difficult searches to conduct, with a considerable tension between wanting to find all relevant materials, and wanting to be subject specific.

Overview

Despite a great deal of concern being publicly expressed over the last decade about unwarranted geographical variation in access to treatment, surprisingly little material was found which reflected on the specific topic of access to NICE-approved therapies.

Effect of an HTA recommendation

Where a health technology appraisal results in a positive recommendation from NICE for the use of the treatment in the NHS, this is very likely to affect the level of usage, although not in all cases. This has been demonstrated through prescribing and sales data.

For example, appraisal TA132 was published in November 2007, recommending the use of ezetimibe and simvastatin/ezetimibe for high levels of cholesterol in the blood. Data from 131 GP practices showed that in the 12 months to September 2008, the number of items prescribed and dispensed for ezetimibe increased by 35.7 % on the previous year (Bibi and Louise, 2010).¹²

Likewise, positive NICE recommendations were associated with an increase in the sales of two medical devices that were studied; but had no influence on a third (Cabo et al, 2011).¹³

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By contrast, however, where NICE has issued negative appraisals these have not always had an impact on practice. In one study relating to 14 such ‘negative or restricting’ recommendations between 2000 and 2004 there was no subsequent reduction in the number of prescription items dispensed and net ingredient costs in the NHS in England and Wales (Dietrich, 2009).¹⁴

Similarly, the effect of TA43, published in June 2002 should have been to limit the prescription of antipsychotic drugs for schizophrenia, particularly by recommending that atypical and typical antipsychotics should in general not be prescribed together. However, one localised study of around 180 inpatients and outpatients found that such co-prescribing had increased between 2004 and 2008 for inpatients (Holland et al, 2009).¹⁵

Limits to implementation

While prescribing and sales data may show increased take-up of NICE-approved treatments, other evidence shows that there are limits to the full acceptance and implementation of HTAs.

There are various examples of HTAs having only a partial effect on local practice. TA49, published in 2002, recommended using ultrasound to assist in placing cannulae into jugular veins. A postal survey of 2000 senior anaesthetists published in 2008, however, showed that only 27% used this as their first choice technique (though they were more likely to use it when teaching students) (McGrattan et al, 2008).¹⁶

A survey of 18 maternity hospitals in the north west of England found that, five years after TA41 on routine antenatal anti-D prophylaxis (RAADP), only 11 had implemented the recommendation (Basu and Bellis, 2007).¹⁷

An evaluation of the implementation of three technology appraisals relating to the use of biologics for severe psoriasis (TAs 103, 134, 146) via 149 patient records in six dermatology centres found that compliance was ‘entirely appropriate’ for the start of biologic therapy, and the dosing. However, where the guidance recommended withdrawing one treatment, etanercept, doctors were keeping it in continuous use (Bewley et al, 2009).¹⁸

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Similarly, an audit of compliance with TAs 104 and 125 on the use of anti-tumour necrosis factor agents for psoriatic arthritis in three hospitals in south west England found 100% compliance with two of the five standards set by NICE, but sub-optimal compliance with the other three (Juarez et al, 2009).¹⁹

In some studies, the level of non-compliance is such that it appears to indicate active resistance to implementation. For example, a survey of general practice in Devon, to which 81 GPs responded, found that, for four of the five technology appraisals in question, NICE guidance in isolation had little impact on GP prescribing, and one had no effect (Wathen and Dean, 2004).²⁰

Similarly, when nephrologists and transplant surgeons throughout the UK were surveyed on their response to TA85 on the use of immunosuppressive therapy for renal transplantation in adults, the conclusion was that “the publication of this NICE guidance has resulted in relatively few changes in prescribing practice: UK transplant centres continue to use a wide range of locally developed protocols for immunosuppressive therapy” (Warren et al, 2008).²¹

Reasons for non-compliance

Most studies did not include detailed examination of the reasons for any limitations to the implementation of these technology appraisal recommendations, but some produced indicative findings. Among the reasons suggested were the following:

- **timing**

The context into which recommendations are released may have a significant effect on implementation. Bibi and Louise (2010) found that, in a clinical context where prescribing was already changing, that trend continued in line with the guidance in the following year. Sheldon et al (2004)²² found that Wisdom tooth extractions fell in line with the 2000 guidance, but had already been falling in the preceding years.

By contrast, unfortunate timing can limit the effect of the recommendations. Sheldon et al (2004) noted how guidance on analogue hearing aids was at first

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enthusiastically received by audiology departments, but at the same time the Department of Health implemented a series of pilots of digital hearing aids, which cut across the guidance on analogue aids. The latter was withdrawn.

- **organisational management proficiency**

Systemic weaknesses in some NHS organisations prevent or delay the implementation of HTA recommendations.

These can be at the level of the clinical unit. For example, Juarez et al (2009) concluded that the reasons for suboptimal compliance with three NICE standards included ‘inadequate clinical documentation, lack of capacity in clinical practice to do timely assessments, and no/erroneous use of PsARC’ (a tool to assess the responsiveness to treatment of the patients). Some of these problems were common to all six centres being studied.

Or they can be at the overall organisational level. The Audit Commission (2005) found serious weaknesses in local financial management arrangements: “Only 26 per cent of NHS bodies participating in this study regularly undertake horizon scanning to assess the financial impact of forthcoming guidance”, and many trusts were weak on estimating the costs and/or savings resulting from technology appraisals.

Sheldon et al (2004), because they studied data across many trusts of various types, found that ‘Some trusts seemed to exhibit more consistent compliance than others across a range of guidance’, and were able to detail their characteristics:

- Commitment to managing process of implementing guidance
- Identification of lead clinician at point of NICE announcement of topic for review
- Proactive assessment of local costs and implications of implementation
- Responsibility for funding and implementation vested in locality-wide group
- Strong clinical governance function appropriately resourced
- Culture of consensus

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- Recognition of legitimacy of NICE
- Involvement of clinicians in guideline process
- Financial stability
- Expectation that compliance is mandatory, subject to identification of funding
- Targeted audit of areas of non-compliance

- **funding**

The Secretary of State's direction to implement technology appraisals is supposed to be backed up with funding for the implementation costs.

In 2012 the government again insisted that there should therefore be 'no financial barriers' to implementation.

DH news release, 28 August 2012

However, the perceptions of NHS bodies and clinicians are often that funding is not available or not adequate. Local practices and hospital units may not be aware of how to find funding for a change of practice.

For example, Seldon et al (2004) found that while audiology departments welcomed the recommendations on analogue hearing aids, the range offered was not extended, because 'funding was described in the interviews as a major impediment to implementation'.

A very high proportion – 85% – of respondents to the Audit Commission's review of the implementation of NICE guidance in 2005 said funding was insufficient. The Commission found that implementation was less likely to be achieved where the recommendations involved 'high capital costs or the appraisal involves expensive drugs or prostheses' (Audit Commission, 2005).²³

- **applying cost-effectiveness criteria to clinical effectiveness**

Hughes and Doheny (2011) provide interesting insights into the tension between national NICE recommendations and the local discussion of funding and implementation.²⁴ They audio-recorded the discussions of a panel of clinicians responsible for funding exceptional cases, in a context where NICE initially stated

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that the expensive cancer drug, Tarceva, was not cost effective – but then changed its position in a final Health Technology Appraisal recommending use when the cost did not exceed that of an alternative product.

The authors note that:

“Guidance that takes time to prepare, is released in stages, and relates to particular disease stages, must be interpreted in the context of particular cases. The case-based panel discourse stands in tension with the standardised population-based recommendations in guidance. Panel members, who based their decisions on the central notions of ‘efficacy’ and ‘exceptionality’, often struggled to apply NICE information on cost-effectiveness to their deliberations on efficacy (clinical effectiveness).”

- **attachment to local clinical practice**

As indicated by the study above, local clinicians, working for what they see as the best interests of their patients, may be unwilling to change practice that they can see is giving some benefit. For example, Bewley et al (2009) suggest that continuing to use etanercept for people with psoriatic arthritis, beyond the point where the recommendation is to withdraw, indicated ‘a reluctance of both patients and clinicians to withdraw an at least partly effective therapy in these refractory patients’.

However, it is not always clear that local clinical practice is well founded. Bhola, auditing compliance with TA42 in a London hospital, found that one of the NICE standards was not fully implemented because some of the relevant patients did not meet diagnostic criteria set locally by the clinicians (Bhola S, 2009).²⁵ Notably, the author recommended the establishment of a written protocol for the latter because ‘in this audit the locally agreed measures were obtained verbally from the consultants’.

- **second-guessing**

Some local clinicians or units may actively question the correctness of the recommendations for the patients in front of them. For example, Wathen and Dean

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(2004) found that only one additional zanamivir inhaler had been prescribed by GPs in North Devon because ‘the recommendations of NICE concerning zanamivir were universally rejected’. This had undermined trust in NICE’s recommendations, which in general were grudgingly followed: ‘NICE guidance in isolation had little impact on GP prescribing. Where the guidance coincided with information from other sources, or personal experience, there was some evidence that technology appraisals triggered an increase in prescribing, but that this was not always sustained.’

The role of Primary Care Trusts

The studies referenced above are illustrative of the challenges of implementing NICE technology appraisals in NHS provider organisations.

However, none of the studies found in our literature searches examined the commissioning or funding policies of Primary Care Trusts. This was disappointing and leaves a hole in the findings.

Primary Care Trusts were, until April 2013, the commissioners of local services. They were the accountable organisations, responsible for ensuring that new NICE-approved treatments were included in local formularies, that funding to support implementation flowed through to providers, and that the quality of care delivered by local providers was monitored for compliance.

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One reason that National Voices wanted to research this topic by using patient views and experience was that failures to provide NICE-approved treatments have sometimes been exposed through investigations and campaigns by patient organisations.

In addition to their own sources of intelligence, including direct contact with patients through support groups, helplines, online fora and email, patient organisations may use other methods to investigate provision.

One of these has been to use freedom of information requests to ask about funding decisions or formulary compliance in Primary Care Trusts. This could be comprehensive in the sense of covering all areas of the country with a mandatory request. However, it could also suffer the same limitations as the datasets examined by the NHS Information Centre – that is, the data might only indicate prescribing levels, rather than usage or the adequacy of provision in relation to local need.

Another method has been to survey providers about their implementation of Health Technology Appraisals. This may be less comprehensive, particularly where significant numbers do not reply to the information request. However, it can give a more granular picture of provision.

In this case study we look at what a National Voices member, the Royal National Institute of Blind People (RNIB), discovered through the latter method and subsequent follow-up.

Ozurdex

In July 2011 NICE published Health Technology Appraisal number 229 with a positive recommendation for the use of dexamethasone intravitreal implants (trade name, Ozurdex) in the NHS.

Ozurdex is used to treat people with occlusion of a retinal vein, described by the website patient.co.uk as follows:

“one of the tiny retinal veins becomes blocked by a blood clot. This means that blood cannot drain away from the retina as easily and there is a backlog of blood in the blood vessels of the retina. This can lead to a build-up of pressure in the blood vessels. As a result, fluid and blood start to leak from the blood vessels, which can damage and cause swelling of the retina, affecting your eyesight.”

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If the swelling happens in the centre of the retina – the macula – this is known as central macular oedema. It can be treated with an Ozurdex implant: a capsule inserted into the eye that releases steroids over time.

In February 2012, seven months after the HTA was published, the RNIB wrote to all Primary Care Trusts and to the 125 hospital trusts providing eye care to ask them about their compliance with TA229.

Of the 80% of trusts who responded, the RNIB judged that 42% had a sub-standard service and 12% had no service.

Among hospital trusts, 37% were providing a restricted service or no service. This contrasted with trusts that had implemented the guidance, and which reported using around 20 implant treatments per month.

Subsequently the RNIB initiated a follow-up campaign to pressure the NHS organisations further.

In responding to the National Voices survey of national patient organisations, the RNIB offered to provide us with further details. In November 2012 the charity reported to us on some of the reasons they had uncovered for the failure to implement the recommendation.

Private communication RNIB to National Voices, 6 November 2012

These included:

- **PCT delay:** Primary Care Trusts which used the ‘three months’ allowed in the directions from the Secretary of State to *make the decision* to implement, rather than to achieve implementation
- **PCT restriction:** in at least one case, the PCT agreed to fund the treatment but place additional criteria to those in the NICE guidance on who could have access to it. The government says local formularies should not ‘second guess’ NICE guidance.
- **PCT mechanism:** PCTs should agree to make the *funding* available to their providers for any treatments used under their NHS contract, but in some cases the PCT had required individual funding decisions to be made. Under this route, the clinician must go through a process of applying to the PCT for an ‘exceptional’

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funding decision. The RNIB noted that in the case of one West Midlands PCT this had probably reduced the expected use of the treatment from 80 patients to ten.

- **PCT and/or hospital trust bureaucracy:** the RNIB found in a number of areas that the treatment had been delayed because the decision had to go through a number of committees and boards. Some of these bodies may get involved in ‘reviewing’ the treatment – with a risk of second guessing the NICE recommendation.
- **Hospital-PCT communication:** the communication between providers and commissioners about funding was often unclear. There were instances where the PCT had agreed funding but the provider trust did not appear to know.
- **Hospital lack of capacity:** here provider trusts were aware of the need to start the treatment but claimed not to have the capacity yet. In some cases this would be planned for the following financial year – implying a delay of nearly two years following the HTA publication.

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Are patients achieving their right?

Patients have the right to receive clinically appropriate treatments approved by NICE, but there is no way to ascertain whether that is happening.

Current national data sets are not adequate. Research literature is sparse. Patient organisations have limited knowledge and understanding.

There is no evidence to suggest that denial of such access is widespread. There is a small amount of evidence that the denial of access does happen locally.

This evidence comes from:

- a limited number of recent case studies, where national patient organisations have investigated specific treatments, such as the RNIB case study reported above;
- some published regional and local audits and evaluations, cited above, which demonstrate that NHS providers have difficulty translating positive NICE recommendations into provision; and
- our survey, wherein patient groups claimed to be directly aware of a minimum of 4,928 cases of denial of treatment.

All of these sources are limited and partial, but they indicate that there may be improvements that can be made in the way the NHS receives NICE recommendations and puts them into clinical practice.

The reasons for lack of access

In many 'postcode lottery' stories, PCTs are reported as actively denying treatments to patients as a result of their discretionary funding decisions. But, as recent government action has re-emphasised, there is no discretion with regard to treatments recommended through NICE technology appraisals, and which are clinically indicated for certain patients.

Responding to the RNIB's survey of hospital trusts, Sir Michael Rawlins, chair of NICE,

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published a trenchant opinion article in the [Health Service Journal](#) stating that ‘quite clearly numerous trusts are acting unlawfully’.

His interpretation of the reason for this was that:

“trusts do not wish to use their resources in this manner. Although they know they are required to make NICE-approved products available, they introduce delaying tactics.”

Sir Michael spoke with all the experience of overseeing the body making and publishing the recommendations. However, on the basis of the limited evidence we have been able to collect, we would have to limit ourselves to the observation that this is one interpretation of the motives behind non-compliance.

A small number of surveys and audits have shown that some clinicians believe themselves empowered to ‘second guess’ NICE recommendations, and/or resist their implementation by cleaving to existing local protocols or beliefs for what is ‘good’ clinical practice.

A larger number of studies have demonstrated that ‘funding’ is commonly cited as a barrier to implementation. The government insists that the funding comes with the recommendation and should not be a barrier.

However, clinicians and others in smaller units within provider organisations may not have a ‘clear view’ of where the funding might be found. Funders may not have effectively communicated its availability.

And there do seem to be genuine cases where the funding is sufficient for the treatment itself but not for associated costs that are required for full implementation (for example, one trust that had implemented TA229 on Ozurdex, and was providing around 20 implants per month, was still negotiating with its PCT for funding to provide follow-up clinics).

Another interpretation of the reasons for failures to provide access would centre on what we earlier identified as “organisational management proficiency”. This would include, for example:

- financial management and planning, including horizon scanning for new guidance (Audit Commission 2005)

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- lack of awareness and dissemination of recommendations through organisations
- ability rapidly to provide the necessary staff, facilities or equipment for implementation
- failure to audit, review and change practice at the clinical level, and
- failure within organisations to tackle areas of non-compliance.

Finally, there appear in some instances to be bureaucratic delays (working the new guidance through committees, for instance) and communication failures between organisations (such as when a PCT has made funding available but the provider and/or its clinicians are not aware of this).

Exerting demand

The government's actions since the publication of 'Innovation, health and wealth' have made use of the 'top-down' or supply-led push for NHS organisations to do what they are legally obliged to do.

Since NHS providers are not vertically accountable to government, these actions bear principally on Primary Care Trusts and their successors, CCGs. The government's presumption appears to be that, as Sir Michael Rawlins wrote, local formulary committees are second-guessing NICE and deliberately refusing to fund the recommended treatments.

They may be correct. However, there is a risk that this route to exerting demand will a) overlook the potentially more significant failings of NHS providers, and/or b) be at best temporarily effective, as PCTs are replaced by new Clinical Commissioning Groups.

In theory, it would be desirable for demand to be exerted from another direction – that is, from patients themselves, empowered to call for their right to be respected.

Given the lack of knowledge, misunderstanding and confusion that we have documented among patient groups and organisations, however, this appears currently to be idealistic. As the RNIB told us:

“I think it is only very rarely that a patient actually knows what treatment they

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should be entitled to and then flags this up. The only ones we've heard from have been as a result of their own consultant telling them -- and you can imagine how often that happens."

RNIB, private communication to National Voices, 6 November 2012

Implications for patients' rights

Healthcare in the UK has rarely proven itself responsive to a patient rights approach. Past experiments to move in that direction, such as John Major's Patient Charter have not been notably successful.

The multi-stakeholder process that produced the NHS Constitution in England did not want to expose the NHS to new sources of litigation by producing a 'lawyer's charter', and so the decision was made to codify patients' rights from existing law and statutory instruments – one of which was the Secretary of State's direction to make NICE-approved treatments available to any patient deemed to be in clinical need.

This discussion paper has shown some of the limitations to that approach. While it appears that many patients must be receiving those treatments, this can only be inferred from the absence of widespread evidence to the contrary. At the same time there is sufficient small scale and localised evidence to be aware that small but significant groups of patients are not able to achieve this right.

A major weakness in this approach to patient rights is that patients themselves are unaware of the right, uncertain about what it means in practice, and easily confused by the overlap with other, non-rights-based aspects of access to treatment.

A second major weakness is that, while the NHS – often heavily nudged by central, top-down action – has to some extent organised itself to deliver the right, it has not organised itself to monitor, test and review the achievement of the right. So nobody knows whether it is making a difference to patients, and if so, how many and which groups.

This is in contrast to those rights that were originally set as 'targets', such as the right to be treated within 18 weeks of referral, or to be seen by a cancer specialist within two weeks of referral. These targets not only forced reorganisation of services, but were

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accompanied by *monitoring mechanisms* so that organisations that were failing to meet them could be identified and held to account.

A third (and to some extent, consequent) weakness relates to redress. If patients are unaware of a right, they cannot seek redress for its denial.

Moreover, redress is not organised to provide clear and specific routes for each patient right. The Handbook to the NHS Constitution (Department of Health, 2012b)²⁶ has multiple sections and appendices on ‘what to do if things go wrong’ that apply generally across all the rights and all NHS organisations. They offer a number of possible alternatives which are only likely to confuse patients. In the case of the right of access to NICE-approved treatments, a single direct route of complaint would be advisable.

It is arguable that these three weaknesses may extend to other patient ‘rights’ such as the right to be involved in discussions and decisions about care and treatment, and the right not to be unlawfully discriminated against.

Implications for the NHS Constitution

In July 2012, following the first phase of a review by the NHS Future Forum, the Secretary of State for Health reported on the Constitution’s impact in its first three years (Department of Health, 2012c).²⁷ He stated that:

“ This report necessarily looks at the performance of the NHS against the rights and pledges codified in the NHS Constitution. In many cases, the evidence is either limited in scope, only partially addresses the effect of the Constitution, or simply alludes to the effects of the Constitution in changing behaviour and organisational culture. What matters most to patients and staff is that the rights and pledges set out in the NHS Constitution are delivered. It is therefore important that a clear evidence base for examining this is established.” [our emphasis]

Following the Future Forum’s lead, his report noted that: “Public awareness of the NHS Constitution is relatively low (27%) and patients are not yet using the Constitution as a means of exercising their rights.”

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This discussion paper has illuminated, for one of those rights, the barriers and limitations in the way.

The NHS Future Forum completed the second phase of its review, on how to strengthen the NHS Constitution, in October 2012. The [Secretary of State](#) accepted its principal recommendations that ‘awareness must be raised dramatically’, and that ‘the NHS Constitution must be given greater traction – especially around what happens when the NHS falls short of people’s rights or expectations’.

The government declared that it would establish an expert group, chaired by a minister, to examine how to give the Constitution more ‘teeth’, and would consult publicly in spring 2013.

In the longer term, the second such review of the Constitution will be due by the middle of 2015.

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Based on the findings of this discussion paper, National Voices suggests that the following actions may help towards ensuring that patients' right to receive NICE-approved treatments can be fully realised.

Change the terminology

NICE health technology appraisals are officially referred to by NICE itself, and therefore by the health system in general, as 'NICE guidance'. But they are only one part of a very wide ranging set of guidance the organisation produces.

This, arguably, contributes significantly to the confusion of patients, patient groups and others about what is at issue when we speak about access to treatments recommended in NICE guidance.

In our survey, for example, many respondents were motivated to participate by their awareness that NICE clinical guidelines were not always being implemented.

The terminology conflates mandatory with non-mandatory guidance. Indeed, since the Health Technology Appraisals are legally mandated with no exceptions it is difficult to regard them as 'guidance'. Via the Secretary of State's direction, the HTA recommendations effectively become 'directions' or instructions to the NHS.



We suggest that NICE, with the Secretary of State's approval, should reclassify its HTAs to reflect this non-negotiable status – for example, as 'technology directions'.

Audit

In our searches for relevant research studies on this topic, a number of the most interesting and practically useful results were regional and local audits of NHS practice in implementing Health Technology Appraisal recommendations.

These audits were able not only to check that the treatment was available, but also

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whether it was being used in ‘full compliance’ with the appraisal. Clearly there were cases where not all aspects of the NICE recommendation were being followed. In some cases the authors were also able to report their recommendations for improvement, and even to report on measures taken to ensure full compliance.

Regional and local audits are likely to be more effective means of dealing with areas of non-compliance than, for example, regulatory action or other ‘enforcement’.



We suggest that the Secretary of State, NHS England and others should consider the case for requiring that compliance with each Health Technology Appraisal should be independently audited within, say, 15 months of publication (that is, one year after the deadline for implementation).

We further suggest that provisions to require all clinicians to participate in these and in national clinical audits should be strengthened throughout the NHS in England.

Data sets

Through the review of the NHS Constitution the government has recognised that it is a priority to establish an evidence base for the achievement of the patient rights in the NHS Constitution.

It should therefore consider what further action can be taken to monitor the achievement of the right to access NICE-approved treatments.

The ‘experimental data’ so far published (NHS Information Centre 2012 and 2013) is a start, but its limitations have been discussed above.

The work to develop these statistics results from a commitment made in 2009, via the Pharmaceutical Price Regulation Scheme (PPRS), an agreement between the Department of Health and the Association of the British Pharmaceutical Industry (ABPI). The commitment is to publish, on an annual basis, metrics for uptake by the NHS in England of a number of medicines positively appraised by the National Institute for Health and Clinical Excellence (NICE).

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The working groups are tripartite – bringing together the Department of Health, industry and NICE.



We make two suggestions here that may aid these workstreams, and help to ensure that when the Secretary of State next reviews the Constitution, in 2015, there is better evidence available:

- audit, as discussed above, can not only clarify the extent of compliance with HTAs, it can also serve up data about the extent of actual usage of the treatments, and reasons for exceptions; and
- national patient organisations, if provided with some resource, are able to mount either surveys of relevant patients, or surveys of NHS organisations, that add to the data picture. While these are unlikely ever to be comprehensive, in the continuing absence of reliable hard data, they can add value to national assessments.

As a third suggestion, to take these forward, the working group on metrics may wish to consider opening discussions with patient organisations on this front.

Redress

Patients who believe they are being denied access to a treatment approved by NICE need to know the simplest, most direct route to seek redress.



We suggest this should be by applying directly to their Clinical Commissioning Group for immediate review.

There should be a **time limit** for the response – perhaps something like the 21-day rule for responding to freedom of information requests.

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Local Healthwatch organisations and Patient Advice and Liaison services (PALs) should be informed and equipped on how to advise patients of this mechanism, particularly by helping them to check whether the treatment they are seeking has been positively recommended by NICE.

This mechanism should be **publicised** during any awareness campaigns that result from the Secretary of State's commitment, following the review of the Constitution, to work with NHS England and others to promote the Constitution.

Clinical Commissioning Groups

From April 2013 NHS England assumed its responsibilities to manage the majority of the NHS budget, by overseeing the NHS commissioning system. The role includes being responsible for upholding and promoting the NHS Constitution.

Locally this will be shared with the Clinical Commissioning Groups that have replaced PCTs. The CCGs will be accountable for implementing the Secretary of State's direction to implement in full the recommendations of NICE technology appraisals.

Patient groups are concerned about this transfer. Half of the national organisations and one third of the local groups who completed our survey said they thought access would be worse or variable as a result.

Partly this may be due to the fact that, so far, many voluntary sector groups are finding it difficult to contact the incipient CCGs or to get responses from them about their plans for future local provision (anecdotal views reported by National Voices members).

This in turn is due to the burden and deadlines that these new organisations are facing as they work to establish themselves.



We suggest that NHS England needs to take early action to assuage these fears by demonstrating that it recognises compliance with HTAs as a priority for the new system, and is making plans to inform, guide and monitor CCGs with regard to implementation.

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The authorisation process for CCGs, which is repeated over time as a check on their performance, should include an element of assessing their adherence to GPG1 (the NICE guidance on best practice in formularies), in particular by:

- timely decisions and implementation of new HTAs
- working with other formularies in the area on implementation
- having clear communication with their providers to remove barriers to implementation.

In developing GPG1, NICE found considerable local variety in the composition, titles and decision making approaches of local formularies. There is also a 'patchwork' of local formularies in many local areas that may not share the same decision making approaches or implementation protocols.

GPG1 includes a number of recommendations for these local formularies to work closely together. Because there is such a variety to cover, however, these recommendations are somewhat vague and general, and in any case are only 'best practice' guidelines.



We therefore suggest NHS England, both as the leader of the commissioning system and as the champion of the NHS Constitution, should consider what actions can be taken in future to regularise the workings of formularies in local areas, including whether there can be a 'single formulary' approach, for example by local authority area.

A research agenda

We cannot know that our right is being achieved unless evidence exists to monitor and assess it.

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The government, NHS England and the NHS Constitution working group should balance the emphasis on top-down ‘compliance approaches’ with more research and investigation into local realities and population needs.

When the Expert Working Group considers how to give the NHS Constitution more ‘teeth’, it should not restrict itself to a narrow focus on complaints and redress but should also look at what is required to create the ‘evidence base’ that the Secretary of State has called for.

The government should work with NICE and NHS England to consider what further research could continue to throw light on the nature of the barriers to implementation.

The factors we have outlined may give a useful starting point for this research. In particular, they point to barriers that arise from poor local communication and lack of understanding, especially among provider trusts and clinicians. It would be useful to have some survey or interview-based research to investigate these factors further.

More potently, the most useful intervention that could help to assess the achievement of our right would be to be able to quantify the need for each specific treatment among local populations. This would assist CCGs and others with their decisions on funding and with their monitoring of compliance; and would provide the data to compare ‘observed’ (actual) provision of a treatment against actual, rather than ‘expected’ need.

There is a powerful system of data collection and analysis of conditions and needs through public health directors and observatories, who will henceforth also be supported by Public Health England. This system will feed into local health and wellbeing strategies via the joint strategic needs assessments.

Patient groups have told us that in their experience the numbers of patients with a condition, and therefore a potential need for a specific treatment, in a given population are often underestimated; and that some groups are excluded from the data entirely because the system does not know about or understand them.

Patient and service user organisations have much to offer to improve this data by, for

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instance, helping Directors of Public Health (DPHs) and Health and Wellbeing Boards (HWBs) to identify specific groups of patients in their area, and researching the size of these groups and their expressed needs.



We suggest that Public Health England, with Directors of Public Health and key voluntary organisations, should consider how, over time, this data can be systematically improved and developed to the point where it might act as baseline data for actual need for HTA-approved treatments.

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About National Voices

National Voices

is the national coalition of health and social care charities in England. We work together to strengthen the voice of patients, service users, carers, their families and the voluntary organisations that work for them. We have more than 150 members with 130 charity members and 20 professional and associate members. Our broad membership covers a diverse range of health conditions and communities and connects with the experiences of millions of people.



National Voices

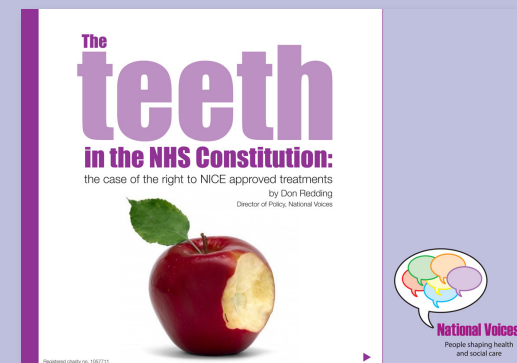
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