Improving UK Patient Outcomes:

How can Value-based Pricing improve access and adoption of new treatments?

Ginette Camps-Walsh
Dr Inge Aivas
Dr Helen Barratt

September 2009

Interim Report:
How should we value new medical treatments?

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ABPI - Association of the British Pharmaceutical Industry
ABHI - Association of British Healthcare Industries
Office of Health Economics
IMS Health
Medical Marketing Group - Chartered Institute of Marketing
About this Publication

How we value medicines and the pricing of new medical treatments, both new medicines and medical technologies, is crucial to the health of the UK. Not only the physical health of the public but also the economical health of the nation and the scientific health of the UK based research and development industry.

This publication was inspired by the political, clinical and industry interest shown in the Office of Fair Trading 2007 report into ‘Value Based Pricing (VBP)’. We wanted to explore further the concept of what ‘Value’ actually means, how VBP is perceived and defined. In order to do this we undertook extensive interviews with stakeholders and had a web-based questionnaire open to all interested parties. This interim report details our findings and makes recommendations based on them.

We are indebted to all our sponsors for their unrestricted funding, on which we depend. As well as enabling our ongoing work of involving frontline professionals in policy ideas and development, sponsorship enables us to communicate with and involve officials and policy makers in the work that we do. Involvement in the work of 2020health.org is never conditional on being a sponsor.

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Improving UK Patient Outcomes

How can value-based pricing improve access and adoption of new treatments?

How should we value new medical treatments?

How can value-based pricing improve patient outcomes?

Executive Summary

This is the interim report for this project, looking at how we value medicines and the pricing of new medical treatments (new medicines and medical technologies). Our second report will cover how we can improve patient outcomes from new treatments, what barriers to uptake exist and how to overcome them, ensuring innovation thrives.

Reviewing the ‘value’ of new treatments

The new Pharmaceutical Price Regulation Scheme (PPRS) 2009 has adopted some of the recommendations from the Office of Fair Trading (OFT) to better reflect the value of new medicines, to the NHS and to patients. This includes pricing flexibilities in certain circumstances, patient access schemes and subject to consultation, generic substitution following the loss of exclusivity after patient protection has elapsed. It introduces post-launch reviews of the ‘value’ of new medicines, conducted by the National Institute for Health and Clinical Excellence (NICE), which could affect the price. It is thought likely that medical technologies will also be assessed in the same way in the future. PPRS also includes patient access schemes for new products. The OFT report on PPRS 2007 suggested that savings of £500 million could be made by introducing ‘value-based pricing’ and substituting old drugs and generics. We argue these savings are not real. Value-based pricing, as described by the OFT, has not worked anywhere in the world. Additionally, they did not consider fully the greater effectiveness and reduced side effects of newer drugs to patients and the potential effect of these on compliance and health outcomes. Prices may, in fact, increase.

How do we value medical treatments?

Since the new PPRS reviews will appraise ‘value’, including ‘value to patients’, how do we value medical treatments?

There is little previous research on this, so 2020Health.org carried out a public consultation with all stakeholder groups to assess what value means to them. There is a large disparity between what the NHS ‘values’ and what taxpayers (who fund the NHS), patients and other stakeholder groups ‘value’. Effectiveness/efficacy and safety rank top, but patients and other stakeholders also consider their dignity, care needed by friends and family, convenience, side effects, time away from work, time to feeling completely well and the invasiveness of treatment as being very important, as well as costs to their employers and the economy. Also, consideration of value for treatments for acute (short-term), chronic (long-term) and end of life disease differ and this should be taken into account.

These attributes need to be considered when valuing medical treatments both by NICE and the NHS.

PPRS

This scheme controls prices and profits of pharmaceutical companies, as the Government strives to negotiate value for money. Pharmaceuticals and medical technologies are reviewed by the Medicines and Healthcare Products Regulatory Agency (MHRA) to ensure their safety and effectiveness before they are licensed for use. NICE appraises products for clinical and cost effectiveness and is unique to England and Wales. Scotland has a different body and methodologies. The high cost of NICE appraisals to companies is not generally appreciated. The additional economic studies to show cost-effectiveness for each product are not required by MHRA or other world-wide licensing bodies. They are estimated to cost an additional £4.5 million for each product, while the appraisals themselves cost an average of £350,000. These are prohibitive for small and medium sized medical companies. This is on top of the average development costs of £530million for a pharmaceutical product. The slow adoption of new medical treatments in the UK, as well as adversely affecting health outcomes, also increases the cost of launching new products in the UK. There is normally only 8-10 years of patent protection left at launch, for a drug, before generic prescribing.

What is value-based pricing?

There is no accepted definition of value-based pricing. Marketers would say that it is pricing at customer’s perceived value (benefits minus costs) and has been in use for many years. Economists have their own definition, especially in respect of medicines: ‘the price that ensures that the expected health benefits exceed the health predicted to be displaced elsewhere in the NHS, due to their additional cost’. However, value-based pricing as defined by OFT in their review of PPRS has never been previously been used.

We interviewed 35 stakeholders about the implications of the new PPRS scheme: patient groups; Government and NHS, including NICE, Department of Health (DH) and OFT; Royal Medical Colleges; academic health economists and policy makers; medical and pharmaceutical trade associations; marketing professionals and pharmaceutical, biotechnology and medical companies.

It was generally felt by non-Government respondents that ‘value’ should reflect value to patients and other stakeholders and many also thought wider costs to society and the economy. Patient access schemes were thought a useful access route by some patient groups, but the risk was borne by the company and not shared. They should be seen as an opportunity to collect more data on the treatment and data on outcomes. In fact collecting data on outcomes from new treatments was cited as a concern. It is very important that a process for this is set up.

It was felt very important, by a significant proportion of those interviewed, that NICE should include wider patient costs in their appraisals and that QALYs (Quality Adjusted Life Years – the assessment used by NICE) should be modified to take this into account. It should also be recognised that different ages, severity of disease, and whether it was acute, chronic, rare or terminal should all be taken into account. One equation does not fit all! While many wanted greater patient participation in NICE appraisals of value, one patient group advocated greater inclusion of clinicians, as they not only understand patient needs, but also the treatments. There was wide agreement among non-government stakeholders that cost effectiveness assessment criteria need to change.

Some saw value reviews as an opportunity to demonstrate the value of a product after launch in order to command a higher price and to show that it would promote medical innovation. Others felt that it would have the opposite effect. Some commented that when a new technology is approved by NICE the NHS is slow to decommission old ones, especially where there is little evidence of clinical effectiveness.
Questions and Recommendations

Questions

- We should have a debate on where we expect our health outcomes to be: On a par with other leading economies? Or (as we suggest) at the leading edge?
- We must then consider the role of health organisations and regulatory bodies in achieving this: Should NICE have its remit extended to help to achieve these health outcomes, taking into account disparities with other leading economies?
- How can value be driven through the system to improve uptake of new treatments and improve patient outcomes?
- Should there be more local scrutiny of PCTs and local health outcomes?
- Should implementation of all NICE guidance be mandatory and enforceable?
- In order to try to accommodate the differing needs of different patients should clinicians be given more flexibility to make decisions about appropriate treatments with their patients?

Recommendations

There should be a public debate on how we value medical treatments.

NICE Technology Appraisal Processes: we recommend the following changes:

- Wider Social and Economic Costs and Benefits Should be included
- The QALY Should be Reviewed
- Categorising Diseases - 5 different categories of disease to be regarded differently:
  - Acute diseases e.g. infections
  - Chronic – long-term disease
  - End of life disease
  - Rare disease
  - Paediatric disease
- NICE thresholds should be reviewed
- Medical technologies and diagnostics should have redesigned appraisal processes
- NICE appraisals should be subsidised, especially for smaller companies
- Price cap increase review
- Explanation of different prices for different indications

Increasing the patent life of medicines should be explored with EU.

Therefore 2020Health.org’s definition of value-based pricing for medical treatments is:

The price that reflects the value to patients, carers, society and the economy which delivers health benefits that exceed the health predicted to be displaced both elsewhere in the NHS and in the welfare economy, due to their additional cost.

The price of a pharmaceutical or medical technology treatment should properly reflect its value.

Using our definition value-based pricing is to be welcomed and should be the basis for pricing for the NHS.

Implications

If this value-based pricing scheme and assessment of ‘value’ is a genuine attempt to give greater rewards to innovative treatments that give significant benefits to patients and improve health outcomes, then it is to be greatly welcomed. However, this would depend on what value the NICE “threshold” (the maximum cost at which it is deemed cost effective) and therefore what price such an innovative beneficial treatment could command.

If this is an attempt at a cost cutting exercise then the consequences are likely to be:

- Higher prices of new treatments at launch to cover higher costs from:
  - Additional NICE appraisals
  - Further delays to adoption and use of new treatments
  - Greater pricing instability, as list prices may be reduced post-launch thus affecting ROI (return on investment)

- Considerably more expensive and bureaucracy for medical companies

- An impact on world prices – as the UK is used for international price benchmarking thus

- Some companies may choose to launch late in the UK, leading to:
  - A reduction in UK clinical trials and research
  - Commercially funded clinical research in UK – may be reduced
  - New treatments would not be available in the UK or be delayed
  - Reduced employment in UK from medical companies
  - Reduced investment in the UK by medical companies

- Parallel Exporting and supply problems for UK patients

- Further delays in patient access to new medical treatments

- A reduction in health outcomes for UK citizens
What is the best way to improve health outcomes by quicker and better patient access to new treatments?

Many factors are responsible for the slower uptake of new treatments in the UK. Most have already been identified although some require more study and international comparison would be interesting.

The Department of Health, NHS, NICE etc processes and procedures are in part responsible for example:

- Payment by Results - the NHS Tariff can discourage uptake of ‘new treatments’ and sometimes there is no established ‘tariff’.
- ‘NICE ‘blight’ and inconsistent uptake of NICE guidance continue to be issues that impact patient access to medicines’ NICE delays access and increases the costs to medical companies, requiring additional evidence.
- The dissemination of information to stakeholders on new treatments and their benefits is inadequate.
- There is a natural conservatism with some clinicians and some vested interests to maintain the status quo.

Finding solutions to the barriers to adoption is pivotal to ensuring patient outcomes can improve from new treatments. These will be considered in our second report.
Chapter Two

2. The NHS: Its objective and goals?

Before we consider how we value medical treatments and pricing of new medical treatments, we should consider why we have the NHS, what we expect from it and what the goals of the NHS should be.

On 5th July 1948, at the launch of the NHS, Aneurin Bevan, the minister of health at the time said (the new NHS), “it had at its heart three core principles:

- that it meet the needs of everyone,
- that it be free at the point of delivery, and
- that it be based on clinical need, not ability to pay”.

The NHS Constitution published in January 2009 states –

The NHS belongs to the people.

It is there to improve our health and well-being, supporting us to keep mentally and physically well, to get better when we are ill and, when we cannot fully recover, to stay as well as we can to the end of our lives. It works at the limits of science – bringing the highest levels of human knowledge and skill to save lives and improve health. It touches our lives at times of basic human need, when care and compassion are what matter most.

The NHS is founded on a common set of principles and values that bind together the communities and people it serves – patients and public – and the staff who work for it.

Principles that guide the NHS: Seven key principles guide the NHS in all it does –

1. The NHS provides a comprehensive service, available to all
2. Access to NHS services is based on clinical need, not an individual’s ability to pay
3. The NHS aspires to the highest standards of excellence and professionalism
4. NHS services must reflect the needs and preferences of patients, their families and their carers.
5. The NHS works across organisational boundaries and in partnership with other organisations in the interest of patients, local communities and the wider population.
6. The NHS is committed to providing best value for taxpayers’ money and the most effective, fair and sustainable use of finite resources.
7. The NHS is accountable to the public, communities and patients that it serves.

And it lists six NHS values:

- Respect and dignity
- Commitment to quality of care
- Compassion,
- Improving lives
- Working together for patients
- Everyone counts

The NHS is funded by individual tax payers and taxes from companies and organisations. Neither the Government nor the NHS has any money of their own. Therefore the NHS should not be put in a silo, but be seen as a much valued provider of healthcare, which along with social care is there for the benefit of citizens, business, the economy and society as a whole. In turn the NHS cannot consider ‘value’ to itself, but must consider ‘value’ to the consumers of healthcare and care.

Despite the current financial downturn the UK is still one of the leading world economies, in approximately 6th place in a world ranking (depending on the criteria used and currency fluctuations etc.), but our health outcomes do not match our economic position. In fact they fall very far short of it. In 2000 the World Health Organisation ranked healthcare in the UK 18th, while our near neighbour, France, was first. More recently cancer survival rates in the UK were found to be not only below the European average, but also ‘similar to survival in some eastern European countries, where the healthcare budget [per capita] is less than a third of that in the UK’.

The standard of the NHS should be to have health outcomes at the leading edge of major world economies.

We have a right to expect health outcomes at least on a par with other leading economies, but by setting standards at the leading edge we would benefit from attracting and retaining the best staff, stimulate research and the UK economy would benefit. A public debate is needed.
From the patient perspective, there are perhaps different spheres of value, which go beyond the health outcomes that dominate the QALY model, to include the true costs or value of a treatment. This might be portrayed in the following way:

There is no doubt that it would be a significant challenge to include more detailed personal costs in the process of valuing a product. At the moment a ‘generic’ patient is considered and the differing priorities and needs of individual patients are ignored. This however raises the issue of whether we should allow for differences in personal circumstances and then include these different considerations and importantly, how would we measure them?

At the same time, the potential benefits need to be balanced with fairness and justice - how do we ensure that costs and benefits are distributed fairly? And what are the most significant benefits? It is important that we do not lose sight of the need for equity in healthcare, ensuring that there is equal provision for equal need. But this then raises the further question of which needs are the most important, and who should decide. And how does all this sit alongside patient autonomy?

Evidence based medicine, appraising the findings of research and applying them to clinical practice to ensure that patients receive the best care, must remain central. Additionally resources are limited and there will always be a need to think about demand management. But alongside this there needs to be public discourse about how priorities are set, and - above all - what really matters to the patient.
3.1. How do we value items we purchase?

Every time we purchase a product or service we make a value judgement. Is our perceived value of the product worth the price? Value is not cost effectiveness. It is specific to each person. Value is an individual perception which compares the benefits with the costs.

People will only purchase a product if their perceived benefits outweigh their perceived costs -

\[ \text{Perceived VALUE} = \text{Perceived benefits} - \text{Perceived costs}. \]

Marketers have a model for valuing products.

The perceived benefits that increase the value and the perceived costs, which detract from the value. These can be broken down into four types:

<table>
<thead>
<tr>
<th>Perceived Benefits</th>
<th>Perceived Costs</th>
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<tbody>
<tr>
<td>Product Benefits</td>
<td>Economic Costs - price</td>
</tr>
<tr>
<td>Service Benefits</td>
<td>Service Costs</td>
</tr>
<tr>
<td>Relational Benefits</td>
<td>Psychological Costs</td>
</tr>
<tr>
<td>Psychological Benefits</td>
<td>Purchasing Effort</td>
</tr>
</tbody>
</table>

Table 1

3.2. Valuing medical treatments

As noted on page 17 the NHS Constitution includes six ‘NHS values’ –

- Respect and dignity
- Commitment to quality of care
- Compassion.
- Improving lives
- Working together for patients
- Everyone counts

However these do not define how we value medical treatments.

Surprisingly there has been very little research on this. This is not the same as the cost-effectiveness of treatment. However there are two studies which touch upon valuing medical treatments: The Oregon Health Plan,\(^{11}\) which started in the late 1980s and a survey by the Kings Fund in 2005.\(^{12}\)

3.2.1. The Oregon Health Plan

In the late 1980s and early 1990s the state of Oregon in the USA consulted their local population on their values and priorities for state funded healthcare. The state government then set priorities for those without health insurance, who would be funded by Medicaid. The 10,000 medical procedures were ranked and priorities set to reduce those funded by the state to 709 medical conditions and treatments known as “condition/treatment pairs”. This was widely commented upon as ‘rationing’ of healthcare, although some dispute this. In a more recent review of this case by Oberlander, Marmor and Jacobs in 2001 for the Canadian Medical Association\(^{11}\) the following observations were made:

‘Through a process of community meetings, public opinion surveys on quality of life preferences, cost–benefits analyses and medical outcomes research, the commission then ranked these condition/treatment pairs according to their “net benefit. These rankings were intended to reflect community priorities regarding different medical conditions and services, physicians’ opinions on the value of clinical procedures and objective data on the effectiveness of various treatment outcomes. … The initial incarnation of the rankings was generated by a mathematical formula that integrated the data from clinicians, the public and outcomes research…. The Oregon approach to rationing, which simultaneously drew on public preferences and cost–benefit analyses, thus represented an unusual marriage of health services research and deliberative democracy.’

The authors cited a number of recommendations and also mentioned the NHS. A few of the more interesting recommendations were:

‘explicit delisting of services is unlikely to produce substantial savings’

‘If cost containment is the goal, implicit rationing, when governments set budgetary caps and limit the supply of costly technology, leaving most so-called bedside rationing decisions to physicians, is a more sound financial strategy for Canadian policy-makers.’

‘Guided by the list, the OHP arguably offers a more sensible Medicaid benefits package today than it did a decade ago, with more emphasis on mental health and preventive services. On the other hand, rationalization of coverage has had little to do with scientific formulae and more to do with the subjective judgements of Oregon’s health administrators. Certainly, research on clinical outcomes and quality of care can enlighten such decisions. Yet ultimately, setting priorities on health care cannot be systematically derived from cost–benefit analyses.’

3.2.2. Kings Fund research on public views on choices in health and health care

This study from 2005 was on patient choice in primary care and not on how patients value medical treatments. Factors influencing patient choice were researched in a number of focus groups and some elements of what they valued in medical treatments were made apparent.

“The choices we make, and the ways in which we make them, are heavily influenced by our personal characteristics, preferences and prior assumptions.”
The value of professional advice and trade-offs between several well-recognised desirable attributes (convenience, quality, continuity and so on) were also evident in treatment choices, both in terms of choosing which treatment to have and in choosing when and where to have it, as the following comments illustrate:

“Oh, I’d want the best person to do the operation. I’d wait for that. There are times when you want the best, and other times when you are happy to have the next down the ladder. It depends how routine it is.”

On a related note, participants made clear distinctions in the way they made choices about different types of health problem. They described judging the severity of their problem, with different strategies for dealing with minor ailments and more serious symptoms. For minor problems that disrupted normal routines, such as a nose bleed or a sprained joint, several participants had used conveniently located, rapid-access clinics (such as walk-in centres) if they were available. 12

It is clear from this study that patients and members of the public view medical treatments in ways that are not routinely considered by the NHS, e.g. convenience. This study was researching attitudes to patient choice, but some of the different aspects of how patients value medical treatments are shown. Their decisions are not made in the way that the NHS makes decisions about treatments. Every patient has different needs and these needs change for different diseases and for their severity.

3.3. 2020Health.org’s Consultation: ‘How do we value medical treatments?’

As little work had been carried out on how the different stakeholder groups value medical treatment, 2020Health.org carried out original web-based research on this.

All stakeholder groups were invited to participate in this public consultation. People were asked to consider how they value medical treatments, using the cost/benefit model [Table 1] from a number of perspectives:

- Patients – individual patients, patient groups, carers, patient advocates
- Medical company managers – pharmaceuticals, medical devices, biotechnology
- Clinicians - NHS hospital doctors, GPs, nurses, pharmacists, technicians, private sector clinicians
- NHS, private sector and DH managers

Respondents were anonymous. The detailed results can be found in Appendix 1.

Respondents

- 35% of respondents were patients, carers or from patient groups
- 24% from medical and pharmaceutical companies
- 29% were clinicians
- 9% NHS and private sector managers

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<th>Benefits</th>
<th>Costs</th>
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<tr>
<td>Effectiveness of the treatment [i.e. does it work]</td>
<td>Long term side effects from treatment - infertility, sleeplessness etc.</td>
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<tr>
<td>Safety of treatment</td>
<td>Complications, readmissions etc. from treatment</td>
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<tr>
<td>Minimal side effects both short &amp; long term</td>
<td>Time to feeling completely well, back to normal, being effective</td>
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<tr>
<td>Making own decisions about treatment</td>
<td>Care needed from friends, family and social services</td>
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<tr>
<td>Outpatient instead of inpatient treatment</td>
<td>Pain, nausea etc - inability to operate as normal</td>
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<tr>
<td>Feeling supported</td>
<td>Time away from work</td>
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<tr>
<td>Becoming knowledgeable about the condition</td>
<td>Fitting treatment into daily routine</td>
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<tr>
<td>Feeling completely well</td>
<td>Loss of dignity and individualism</td>
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<tr>
<td>Expert medical backup 24/7</td>
<td>Being patronised &amp; not being in control/helplessness</td>
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<tr>
<td>Minimally invasive - alternatives to major surgery etc.</td>
<td>Pain / discomfort and short term effects of treatment</td>
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<tr>
<td>Personalised medicine to suit patients e.g. Herceptin only works on</td>
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<tr>
<td>some patient types, some drugs work better on some people than others</td>
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</tr>
</tbody>
</table>

It is clear from this study that patients and members of the public view medical treatments in ways that are not routinely considered by the NHS, e.g. convenience. This study was researching attitudes to patient choice, but some of the different aspects of how patients value medical treatments are shown. Their decisions are not made in the way that the NHS makes decisions about treatments. Every patient has different needs and these needs change for different diseases and for their severity.

3.3. 2020Health.org’s Consultation: ‘How do we value medical treatments?’

As little work had been carried out on how the different stakeholder groups value medical treatment, 2020Health.org carried out original web-based research on this.

All stakeholder groups were invited to participate in this public consultation. People were asked to consider how they value medical treatments, using the cost/benefit model [Table 1] from a number of perspectives:

- Patients – individual patients, patient groups, carers, patient advocates
- Medical company managers – pharmaceuticals, medical devices, biotechnology
- Clinicians - NHS hospital doctors, GPs, nurses, pharmacists, technicians, private sector clinicians
- NHS, private sector and DH managers

Respondents were anonymous. The detailed results can be found in Appendix 1.

Respondents

- 35% of respondents were patients, carers or from patient groups
- 24% from medical and pharmaceutical companies
- 29% were clinicians
- 9% NHS and private sector managers

<table>
<thead>
<tr>
<th>Benefits</th>
<th>Costs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Effectiveness of the treatment [i.e. does it work]</td>
<td>Long term side effects from treatment - infertility, sleeplessness etc.</td>
</tr>
<tr>
<td>Safety of treatment</td>
<td>Complications, readmissions etc. from treatment</td>
</tr>
<tr>
<td>Minimal side effects both short &amp; long term</td>
<td>Time to feeling completely well, back to normal, being effective</td>
</tr>
<tr>
<td>Making own decisions about treatment</td>
<td>Care needed from friends, family and social services</td>
</tr>
<tr>
<td>Outpatient instead of inpatient treatment</td>
<td>Pain, nausea etc - inability to operate as normal</td>
</tr>
<tr>
<td>Feeling supported</td>
<td>Time away from work</td>
</tr>
<tr>
<td>Becoming knowledgeable about the condition</td>
<td>Fitting treatment into daily routine</td>
</tr>
<tr>
<td>Feeling completely well</td>
<td>Loss of dignity and individualism</td>
</tr>
<tr>
<td>Expert medical backup 24/7</td>
<td>Being patronised &amp; not being in control/helplessness</td>
</tr>
<tr>
<td>Minimally invasive - alternatives to major surgery etc.</td>
<td>Pain / discomfort and short term effects of treatment</td>
</tr>
<tr>
<td>Personalised medicine to suit patients e.g. Herceptin only works on</td>
<td></td>
</tr>
<tr>
<td>some patient types, some drugs work better on some people than others</td>
<td></td>
</tr>
</tbody>
</table>

Table 2
What do medical company managers – pharmaceuticals, medical devices, biotechnology value?

The top ten benefits and costs are mentioned here.

**Benefits**
The most important attributes are very similar to the patient group of effectiveness, efficacy and safety. The next highest rankings are all concerned with benefits for patients and outcomes.

**Costs**
The very high costs of R&D in pharmaceuticals, biotechnology and medical devices are well know so this obtained top ranking, followed by clinical trials and manufacturing costs. What might appear more surprising is the ranking of registration costs, clinical education and the cost of both additional economic studies for NICE and the appraisals themselves.

<table>
<thead>
<tr>
<th>Benefits</th>
<th>Costs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Effectiveness of the treatment</td>
<td>Cost of research</td>
</tr>
<tr>
<td>92.1%</td>
<td>60.5%</td>
</tr>
<tr>
<td>Efficacy of the treatment</td>
<td>Cost of clinical trials</td>
</tr>
<tr>
<td>81.6%</td>
<td>55.3%</td>
</tr>
<tr>
<td>Safety of treatment</td>
<td>Manufacturing costs</td>
</tr>
<tr>
<td>81.6%</td>
<td>52.6%</td>
</tr>
<tr>
<td>Improving health outcomes</td>
<td>Cost of registration/CE marking</td>
</tr>
<tr>
<td>76.3%</td>
<td>42.1%</td>
</tr>
<tr>
<td>Improving patients’ quality of life</td>
<td>Marketing &amp; sales costs</td>
</tr>
<tr>
<td>71.1%</td>
<td>42.1%</td>
</tr>
<tr>
<td>Number of patients who could benefit</td>
<td>Pressure on pricing</td>
</tr>
<tr>
<td>63.2%</td>
<td>42.1%</td>
</tr>
<tr>
<td>Side effect profile</td>
<td>Clinician education</td>
</tr>
<tr>
<td>Conveniences of treatment for patients</td>
<td>NICE appraisals</td>
</tr>
<tr>
<td>60.5%</td>
<td>42.1%</td>
</tr>
<tr>
<td>Shorter hospital stay</td>
<td>Costs of economic studies for NICE</td>
</tr>
<tr>
<td>52.6%</td>
<td>39.5%</td>
</tr>
<tr>
<td>Treating patients more effectively</td>
<td>Opportunity costs of investment</td>
</tr>
<tr>
<td>52.6%</td>
<td>36.8%</td>
</tr>
</tbody>
</table>

Table 3

<table>
<thead>
<tr>
<th>Benefits</th>
<th>Costs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Effectiveness of the treatment (i.e. does it work)</td>
<td>Cost savings as a result of better results of treatment</td>
</tr>
<tr>
<td>95.8%</td>
<td>54.2%</td>
</tr>
<tr>
<td>Efficacy of the treatment (i.e. beneficial effect)</td>
<td>Severity of the disease</td>
</tr>
<tr>
<td>63.3%</td>
<td>39.6%</td>
</tr>
<tr>
<td>Good long term safety profile</td>
<td>Costs of diagnostic tests required to monitor drug safety</td>
</tr>
<tr>
<td>72.9%</td>
<td>31.3%</td>
</tr>
<tr>
<td>Improving Patient outcomes</td>
<td>Informing patients</td>
</tr>
<tr>
<td>70.8%</td>
<td>31.3%</td>
</tr>
<tr>
<td>Good research evidence that it is effective 68.8%</td>
<td>Saving on NHS tariff</td>
</tr>
<tr>
<td>Low side effect profile</td>
<td>29.2%</td>
</tr>
<tr>
<td>More effective management of patient</td>
<td>Persuading local formulary committees/managers</td>
</tr>
<tr>
<td>47.9%</td>
<td>27.1%</td>
</tr>
<tr>
<td>Supporting patients’ treatment decisions</td>
<td>Changing treatment protocols</td>
</tr>
<tr>
<td>43.8%</td>
<td>20.8%</td>
</tr>
<tr>
<td>Shorter hospital stay</td>
<td>Risk management changes</td>
</tr>
<tr>
<td>43.8%</td>
<td>20.8%</td>
</tr>
<tr>
<td>Primary care vs. outpatient vs. inpatient treatment</td>
<td>Increased diagnostic tests</td>
</tr>
<tr>
<td>35.4%</td>
<td>20.8%</td>
</tr>
<tr>
<td></td>
<td>Training staff</td>
</tr>
<tr>
<td></td>
<td>18.8%</td>
</tr>
</tbody>
</table>

Table 4

What do clinicians - NHS hospital doctors, GPs, nurses, pharmacists, technicians and private sector clinicians - value?

The top ten benefits and costs are mentioned here.

**Benefits**
Those ranked top were consistent with the other stakeholder analyses: effectiveness/efficacy and safety. Attributes concerning the product/treatment profile, good research evidence and low side effects were also ranked as very important. Looking at the benefits to the patient, improved outcomes ranked highest, as well as more effective treatment and supporting patients’ treatment decisions, which also ranked very highly with patients.

**Costs**
Many of the costs nearer the top of the list were connected with changes in treatment protocols. None were mentioned that were specifically patient-centred.

<table>
<thead>
<tr>
<th>Benefits</th>
<th>Costs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Effectiveness of the treatment</td>
<td>Cost savings as a result of better results of treatment</td>
</tr>
<tr>
<td>95.8%</td>
<td>54.2%</td>
</tr>
<tr>
<td>Efficacy of the treatment</td>
<td>Severity of the disease</td>
</tr>
<tr>
<td>63.3%</td>
<td>39.6%</td>
</tr>
<tr>
<td>Safety of treatment</td>
<td>Costs of diagnostic tests required to monitor drug safety</td>
</tr>
<tr>
<td>81.6%</td>
<td>31.3%</td>
</tr>
<tr>
<td>Improving health outcomes</td>
<td>Informing patients</td>
</tr>
<tr>
<td>76.3%</td>
<td>31.3%</td>
</tr>
<tr>
<td>Improving patients’ quality of life</td>
<td>Saving on NHS tariff</td>
</tr>
<tr>
<td>71.1%</td>
<td>29.2%</td>
</tr>
<tr>
<td>Number of patients who could benefit</td>
<td>Persuading local formulary committees/managers</td>
</tr>
<tr>
<td>63.2%</td>
<td>27.1%</td>
</tr>
<tr>
<td>Convenience of treatment for patients</td>
<td>Changing treatment protocols</td>
</tr>
<tr>
<td>60.5%</td>
<td>20.8%</td>
</tr>
<tr>
<td>Side effect profile</td>
<td>Risk management changes</td>
</tr>
<tr>
<td>63.2%</td>
<td>20.8%</td>
</tr>
<tr>
<td>Number of patients who could benefit</td>
<td>Increased diagnostic tests</td>
</tr>
<tr>
<td>63.2%</td>
<td>20.8%</td>
</tr>
<tr>
<td>Conveniences of treatment for patients</td>
<td>Training staff</td>
</tr>
<tr>
<td>60.5%</td>
<td>18.8%</td>
</tr>
</tbody>
</table>

Table 4
Ranking of attributes to be considered when valuing new medical treatments

All respondents were then asked to rate the different benefits and costs by their importance in measuring the value of a new medical treatment. The percentage responses given the highest rating ‘extremely important–must be considered’ are shown below in Table 6. The first two receiving the highest rating are the same as in the individual stakeholder group analyses: ~89% said that effectiveness/efficacy was extremely important, while 9% thought it was very important. This was closely followed by safety, which 68% thought extremely important and 26% very important. ‘Health outcomes – successfulness of treatment’ ranked next with 63% rating it extremely important and 27% very important.

Lossofdignityandindividualismwas thenext most highly rated with 25% thinking it extremely important and 32% very important. Complications and readmissions, side effects and cost to the patient were rated next.

None of the parameters were seen as unimportant.

Now thinking as an individual citizen, how would you measure the value of a medical new treatment? Please rate the parameters above from 1 to 6, 1 being most important, 6 being least important.

## Table 5

<table>
<thead>
<tr>
<th>Benefits</th>
<th></th>
<th>Costs</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Effectiveness of the treatment [i.e. does it work]</td>
<td>83.3%</td>
<td>Price to NHS</td>
<td>73.3%</td>
</tr>
<tr>
<td>Safety of treatment</td>
<td>73.3%</td>
<td>NHS staff resource requirements</td>
<td>56.7%</td>
</tr>
<tr>
<td>Health outcomes</td>
<td>73.3%</td>
<td>Longer-term costs, social care etc</td>
<td>59.0%</td>
</tr>
<tr>
<td>Severity of the disease</td>
<td>50.0%</td>
<td>Patient pathway costs</td>
<td>46.7%</td>
</tr>
<tr>
<td>No/less long-term side effects</td>
<td>50.0%</td>
<td>NHS tariff</td>
<td>43.3%</td>
</tr>
<tr>
<td>Easy/convenient dosage/treatment regime</td>
<td>40.0%</td>
<td>Complications, readmissions etc</td>
<td>36.7%</td>
</tr>
<tr>
<td>Local care near or at home</td>
<td>26.7%</td>
<td>Staff training</td>
<td>36.7%</td>
</tr>
<tr>
<td>Speed of action</td>
<td>23.3%</td>
<td>Diagnostic &amp; equipment costs</td>
<td>33.3%</td>
</tr>
<tr>
<td>Health outcomes – successfulness of treatment</td>
<td>30.0%</td>
<td>Patient information</td>
<td>33.3%</td>
</tr>
<tr>
<td>Convenience of treatment for patient i.e. tablet once a day at home</td>
<td>28.7%</td>
<td>Wating times</td>
<td>20.0%</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Table 6</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Extremely Important - must be considered</td>
<td></td>
</tr>
<tr>
<td>Effectiveness &amp; efficacy of the treatment [i.e. does it work]</td>
<td></td>
</tr>
<tr>
<td>Safety of treatment</td>
<td></td>
</tr>
<tr>
<td>Primary Care vs. outpatient vs. inpatient...</td>
<td></td>
</tr>
<tr>
<td>Care needed from friends and family and social care</td>
<td></td>
</tr>
<tr>
<td>Complications, re-admissions and costs of these</td>
<td></td>
</tr>
<tr>
<td>Side effect profile - few or many negative side effects</td>
<td></td>
</tr>
<tr>
<td>Health outcomes of treatment - successfulness of treatment</td>
<td></td>
</tr>
<tr>
<td>Convenience of treatment for patient i.e. tablet once a day at home</td>
<td></td>
</tr>
<tr>
<td>Ability to treat more severe or new diseases, especially rare ones</td>
<td></td>
</tr>
<tr>
<td>Patient time to feeling completely well/return to work</td>
<td></td>
</tr>
<tr>
<td>Cost to the economy - non-productivity/sickness benefit etc.</td>
<td></td>
</tr>
<tr>
<td>Costs to employer of sickness absence etc.</td>
<td></td>
</tr>
<tr>
<td>NHS price and treatment cost</td>
<td></td>
</tr>
<tr>
<td>Invasiveness of treatment e.g. alternatives to major surgery</td>
<td></td>
</tr>
<tr>
<td>Loss of dignity and individualism</td>
<td></td>
</tr>
<tr>
<td>Cost of clinical trials &amp; economic studies for NICE</td>
<td></td>
</tr>
<tr>
<td>Cost of registration/CE marking &amp; post launch surveillance</td>
<td></td>
</tr>
<tr>
<td>Marketing, sales, launch &amp; distribution costs</td>
<td></td>
</tr>
<tr>
<td>Cost to patient - side effects of treatment e.g. nausea, travel etc.</td>
<td></td>
</tr>
<tr>
<td>Research, development &amp; manufacturing costs</td>
<td></td>
</tr>
</tbody>
</table>

Table 6
If a ranking is performed by attributing numerical values to each rating i.e. ‘extremely important’ = 6 through to ‘should not be considered’ = 1, an overall ranking can be obtained; this is shown below in Table 7.

Those which the NHS and NICE do not seem to consider but which ranked highly are:

- Patient time to feeling completely well/ returning to work
- Loss of dignity and individualism
- Costs to patient of side effects
- Invasiveness of treatment
- Care needed from friends and family
- Where they are treated i.e. hospital, primary care, home
- Convenience of treatment
- Costs to the economy
- Costs to employer

In view of this it is now time that the NHS and NICE considered these costs to those who are funding the NHS and not just the cost to the NHS and social services in isolation.

### Table 7

<table>
<thead>
<tr>
<th>Ranking</th>
<th>Value</th>
<th>Weighting</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Effectiveness &amp; efficacy of the treatment [i.e. does it work]</td>
<td>748</td>
</tr>
<tr>
<td>2</td>
<td>Safety of treatment</td>
<td>702</td>
</tr>
<tr>
<td>3</td>
<td>Health outcomes of treatment - successfulness of treatment</td>
<td>683</td>
</tr>
<tr>
<td>4</td>
<td>Side effect profile - few or many negative side effects</td>
<td>604</td>
</tr>
<tr>
<td>5</td>
<td>Complications, readmissions and costs of these</td>
<td>585</td>
</tr>
<tr>
<td>6</td>
<td>Patient time to feeling completely well/return to work</td>
<td>574</td>
</tr>
<tr>
<td>7</td>
<td>Loss of dignity and individualism</td>
<td>561</td>
</tr>
<tr>
<td>8</td>
<td>Cost to patient - side effects of treatment e.g. nausea, travel etc.</td>
<td>555</td>
</tr>
<tr>
<td>9</td>
<td>Invasiveness of treatment e.g. alternatives to major surgery</td>
<td>554</td>
</tr>
<tr>
<td>10</td>
<td>Ability to treat more severe or new diseases, especially rare ones</td>
<td>554</td>
</tr>
<tr>
<td>11</td>
<td>Care needed from friends and family and social care</td>
<td>534</td>
</tr>
<tr>
<td>12</td>
<td>Primary Care vs. outpatient vs. inpatient - where treated - home, GP, hospital &amp; length of hospital stay etc.</td>
<td>527</td>
</tr>
<tr>
<td>13</td>
<td>NHS price and treatment cost</td>
<td>524</td>
</tr>
<tr>
<td>14</td>
<td>Convenience of treatment for patient i.e. tablet once a day/at home</td>
<td>523</td>
</tr>
<tr>
<td>15</td>
<td>Cost to the economy - non-productivity/sickness benefit etc.</td>
<td>512</td>
</tr>
<tr>
<td>16</td>
<td>Costs to employer of sickness absence etc.</td>
<td>497</td>
</tr>
<tr>
<td>17</td>
<td>Research, development &amp; manufacturing costs</td>
<td>447</td>
</tr>
<tr>
<td>18</td>
<td>Cost of clinical trials &amp; economic studies for NICE</td>
<td>436</td>
</tr>
<tr>
<td>19</td>
<td>Cost of registration/CE marking &amp; post launch surveillance</td>
<td>421</td>
</tr>
<tr>
<td>20</td>
<td>Marketing, sales, launch &amp; distribution costs</td>
<td>376</td>
</tr>
</tbody>
</table>

Respondents were then asked to comment upon whether the ‘value’ should be changed in respect of acute (short-term) illness versus chronic (long-term illness) versus end of life illness.

### Comments on short-term illness

The example given for a short-term illness was flu, although an acute infection would also have come into this category. There was no real consensus on this. While some felt that they would be prepared to put up with more side effects for a short-term treatment, some were not prepared to suffer any side effects at all. Others felt that flu did not need any treatment at all and people should ‘tough it out’. Compliance was mentioned as an important factor; if there are side effects to the treatment compliance will be affected. All the comments can be found in Appendix 8. Here is a sample:

- Unpleasant side effects can be tolerated if there is a short time period and an end point value for short term conditions is speed of treatment
- I may be more tolerant of side effects but to be honest I don’t want any short or long term
- Would choose the drug with least side effects
- Rapid recovery from a minimally invasive technique as long as it’s safe and effective is very important
- Provision of care for the patient during this period. Ability of patient to work during this period

### Comments on long-term illness

The example given for a long-term illness was diabetes. The comments were very varied. Some made the point that safety and efficacy where the most important issues, but most agreed that side effect profile is important. Again comments were made about compliance and quality of life. The full list of comment can be found in Appendix 8. Examples of these include:

- Treatment, effectiveness, ease of use, ease of access and costs important this should be tailor made for the patient and where possible the person should have independent care in their homes
- Inconvenience would be important, but survival more so
- Want effective medication that enables me to continue working for a living
- I would put up with some inconvenience to improve quality of life the device is very important + one injection a day the better
- The simpler the better for the dose regime as people are so busy they regularly forget to take their medicine?
- For a long term condition it is vital that possible side effects from years of use are made known to patient and family, and that such medicines are removed from ‘repeat’ when patient has recovered
- Impact on daily life is very important. Ability to live life as if I didn’t have the condition would be important.
- A product for a long term condition would be of greater value the more seamless it would be to fit into existing lifestyle, for example an inhaler that lasts longer, is more portable, technology that reduces inconveniences of regular face to face discussions with a medical professional etc. The least side effects the better as this is not something you want to get used to.
Comment on terminal illness

Most would consider putting greater value on effectiveness and would put up with more side effects for a terminal illness such as cancer. One commented that - “for terminal diseases value is pain free life extension”. Another said that ‘everything that is possible should be done for people with cancer with no regard for cost’.

The NHS and NICE might not agree with this approach. Here were many comments on maintaining dignity.  

- If product saves lives or seriously improves quality of life when has been very poor, worthwhile, but only to a limit - probably quite individual
- It is for the patient to decide whether the benefits outweigh the risks based on as accurate information as possible including data from negative trials
- Patients’ dignity in dying. Support and guidance available.
- Value is speed of DX (diagnosis) and access to optimum Tx (treatment) to maximise outcomes

How would you ensure delivery of good value medical treatments now and in the future?

Again there were many interesting and novel comments. Most did agree that patients should be involved. Some wanted to see alternatives to NICE, but most felt that NICE should oversee this process, but with more involvement from patients and other stakeholders. However, there was some feeling that doctors are best placed to make the decisions either instead of NICE or with a greater role within it. Some of the comments are shown here:

- The price should be set by the government, cheap loans could be given by the government to cover R&D costs to cover companies until the treatment is used sufficiently within the population to cover production costs
- Government should stop interfering. Leave it with the experts
- Allow pharma companies to get more involved in healthcare delivery
- GP should remain in control of these decisions on an individual basis
- Co-pay for more low cost treatments to increase funds to high cost new drugs
- Patients should always be treated as individuals and their wishes and those of their carers should be taken into consideration. There must be informed consent to any treatment.
- Having a government body which is totally removed from the needs of the individual and which only looks at cost of treatment is not desirable

Summary and conclusions from the survey

The PPRS letter mentioned ‘value to patients’, although this seems to be absent from subsequent documents. However, as tax payers are paying for the NHS and it is not free it is important, indeed absolutely necessary, that patient values are taken into account. What is the NHS for, if not for patients?

This consultation gives new insight into the different values important to different stakeholder groups, including patients, medical companies, clinicians and NHS/healthcare managers.

While the benefits most highly valued by all groups are effectiveness/efficacy and safety, which NICE and the NHS evaluate at present, other highly rated costs and benefits are not widely considered, or considered enough.

While costs to the NHS are always considered, costs to patients, their families, their employers and society are not, although they fund the NHS. These costs can be significant and in a publicly funded, or indeed any healthcare system must be taken into account –

- Patient time to feeling completely well/ returning to work
- Loss of dignity and individualism
- Costs to patient of side effects
- Invasiveness of treatment
- Care needed from friends and family
- Where they are treated i.e. hospital, primary care, home
- Convenience of treatment
- Cost to the economy
- Cost to employer

Different values were attributed to acute, chronic and end-of-life treatments and these should be viewed differently, when assessing value, as side effects for chronic disease are more important than those for the other two categories.

To ensure that value is delivered both now and in the future most, but not all, thought that NICE should oversee this process, but that patients and doctors should be much more involved in the process.

When assessing the ‘value’ of new or even existing treatment the NHS, NICE, the Department of Health (DH) and the Government, must take into account the costs to patients, their families, society, their employers and the economy. The NHS is not in a silo. It is there for society and the economy.
4.1. How are pharmaceuticals and medical devices priced?

The pricing of pharmaceuticals, biotechnology products and medical technologies/devices are very complex and viewed on a global basis. A simple overview is given here, which shows some of the major considerations.

Pharmaceutical, biotechnology and medical technology/device companies already price their products on what might be called a value-based system. A considerable amount of research is carried out on how their customers would value the products and how they assess the benefits and costs. Some medical device companies still price on a cost plus basis, as their life cycles and profit margins vary more widely.

Prices must obviously cover all costs and make a profit to invest in future development.

The Cost of Bringing a Pharmaceutical Product to Market

Approximately 10,000 molecules are researched and developed to enable one new pharmaceutical product to be launched. The development process goes through laboratory testing and three phases of clinical trials to prove safety and effectiveness. This process costs on average £550 million and takes 10 to 12 years. A patent is taken out when the molecule is first discovered, so by the time the product is launched, there is only 8-10 year’s sales to recoup costs before it goes off patent and generic competition begins.

If a new product successfully passes through all the development phases and clinical trials it must gain marketing authorisation, not from the NICE, but from the Medicines and Healthcare Products Regulatory Agency (MHRA) or the European Medicines Evaluation Agency (EMEA) for biotechnology products, certain diseases and new active substances. These agencies decide whether a product is safe and effective enough to be launched. They review all the clinical evidence. The cost of a license is high. MHRA charge £104,108 for a full license and EMEA charges €251,600.
The regulatory requirements are being harmonised across the world, so all are becoming similar to increase quality standards and reduce additional country specific clinical trials and costs.

After launch the product is monitored for safety by the company and government agencies MHRA. This process is known as pharmaco-vigilence.

The cost of bringing a medical device/technology product to market
The development process for medical devices and technologies are very varied as the complexity of products varies from simple dressings to very complex indwelling devices such as pacemakers and drug eluting stents. There are also sophisticated electronics such as MRI scanners and gamma knives for neurological procedures. These can have development lead times approaching pharmaceuticals or be much shorter.

The MHRA will also establish safety and effectiveness in a similar way to pharmaceuticals, with a CE mark being awarded.

**Should prices be higher in the UK?**
For pharmaceuticals, medical devices and biotechnology products the UK has higher costs and lower sales per head than other developed markets e.g. Europe and USA. There are four main reasons for this –

- In the UK we spend less on health as percentage of GDP than most other leading economies
- In the UK we spend less on medicines per capita than other leading economies
- In the UK we spend less of a percentage of our total healthcare expenditure on medicines and medical technologies than other leading economies
- The UK is one of the slowest developed countries to adopt new medical treatments

- NICE assessments are very expensive –
  - additional economic trials
  - the process itself
  - delays while awaiting NICE decisions delay adoption further

The reason for the delays in adopting new treatments in the UK will be researched in the second part of our report.

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**Total expenditure on health as a percentage of GDP**
The UK has one of the lowest expenditures on health as a percentage of GDP. USA has 16%, France 11%, Germany 10.4%, Belgium 10.2%, and Austria 10.1%. Of leading EU and USA economies only Ireland at 7.6% and Finland at 8.2% have lower spending than the UK at 8.4% of GDP. Source OECD from 2007 (report 2009) (Japanese figures not available).

**Per Capita Expenditure on Medicines**
In the UK we spend £200 per person each year on medicines, whereas in Germany they spend £269, France £372, and USA £507. A company will need to gain a return on their investment when launching a new treatment and the much lower expenditure on medicines in the UK means potentially lower sales per capita and therefore would normally command a higher price...

**Percentage of UK healthcare budget spent on pharmaceuticals and medical technologies**
In the UK we spend under 12% of our total healthcare budget on medicines according to the ABPI. According to OECD (Health data 2009) Austria spends – 13.3%; Belgium– 15.7%; France - 16.3%; Germany – 15.1%; Italy – 19.3%; Spain- 21% and Sweden – 13.4%.
Cost of NICE assessments
In order to go through a NICE appraisal process a company will have to carry out complex and expensive economic clinical trials to prove cost-effectiveness. In addition putting together a submission for NICE is very expensive. The ABPI estimates that it costs an average of £325,000 (range £116k - £600k) for the assessment alone and £4.5million to carry out the additional cost-effectiveness trials for the NICE Technology Appraisal. This put a considerable additional financial burden on companies going through this process of NICE appraisal in the UK. For small companies it is absolutely prohibitive and could completely stifle new treatments coming to market. In practice many will try to avoid NICE appraisals, even if they could be positive and support use of the new treatment.

Costs of slow adoption and uptake of new medical treatments in the UK
It is not possible to assess the cost of the slow uptake and adoption of new medical treatments in the UK. The costs are manifold: cost to patients; lives lost that might have been saved or extended; lower quality of life; costs to the economy in terms of working days lost, etc.; costs to medical companies in terms of revenue to offset research, etc.

The patent life of a pharmaceutical product at launch is only 8 to 10 years; for medical devices it varies, depending on the development required. This is because the patent is taken out at an early stage of development. The company therefore has a relatively short time to recoup the £550 million spent on research before generic competition is introduced. It is Government and Conservative Party policy to retain generic prescribing. Similarly with medical devices PASA (NHSCoaching and Supply Agency) puts products out to tender and tries to turn them into commodities as soon as possible, to reduce the price. The margins on medical devices are usually lower than pharmaceuticals.

After marketing authorisation (from MHRA) a company needs to invest considerable amounts of extra money in launching, marketing and selling the new product. Much of this expenditure is on education and training of medical, nursing and technical staff to persuade them to use it effectively and safely.

These factors make it considerably more expensive to launch a product in the UK. The additional costs and delays involved with NICE assessments and value assessments would raise costs and therefore potentially prices in the UK.

Value-based pricing
There is no accepted definition for value-based pricing and this will be discussed in more details later. However, taking the OFT version as a model there are the following observations.

Firstly most pricing models start with the highest product price at launch, to reflect ‘value’ and recoup R&D and launch costs. The price gradually erodes over time due to inflation, competition, new innovations and changes in the market. Very few prices increase in real terms after launch, unless raw materials are in short supply.

Secondly, a company will analyse, forecast and assess the return on investment (ROI) before investing in developing and launching products. Value based pricing would give too great an uncertainty as to the return. Some companies have already warned, in recent published interviews, that they may not launch new products in the UK. It is possible that others would follow suit. This would not only deprive UK patients of new technologies and treatments and therefore potentially reduce UK health outcomes, but also jeopardise jobs, exports, tax revenue and investment in the UK.

Thirdly, some countries in Europe now compare market prices across major countries to ensure consistency as a matter of routine. If pricing were lower in the UK, this would have a serious adverse effect on the returns for pharmaceutical and medical companies across Europe and even worldwide. This is a major disincentive for companies to launch new medicines and medical technologies in the UK.

4.2. Pricing regulation for pharmaceuticals
Pricing regulations have been used by successive Governments to protect consumers in markets where suppliers, such as utility companies, have a monopoly on supply. It is difficult for a member of the public to try to negotiate a lower price for their water bill for instance, or to negotiate a train fare.

In a free market with competition and free customer choice, there should be no need for price regulation. This could describe the market for medical treatments: pharmaceuticals, medical devices and medical technologies. There is competition in this market and the NHS is almost a monopoly purchaser, so is there any need for price regulation?

As described previously there is a disconnection between funding, decision making on purchasing and prescribing and the consumer [the patient] in healthcare, so expenditure management is difficult without external control.

Several studies challenge this notion of free competition and this will be discussed later in the report.

Most governments, however, negotiate pricing with medical companies if they have a state funded healthcare system. Most governments also have some sort of pricing control, the rationale being that they are seeking ‘value for money’ for the tax payer. In countries with private or insurance based healthcare system the insurance companies will negotiate prices with medical companies.

In the past two decades there has been an increase in pharmaceutical expenditure as a percentage of total healthcare expenditure in OECD countries, from 13% in 1980 to 18.1% in 2003. This forms the basis for cost containment policies, acceptable to both politicians and to a healthy economy.

Pharmaceutical pricing regulation usually takes into account two main objectives: those of society and those of industry. Since UK healthcare expenditure is funded by tax payer, governments must be efficient in purchasing medicines and other medical treatments. They must ensure that medicines are available both to the NHS and the individual. Since the welfare of the population is the ultimate goal of the government, it must strive for medical innovation (to improve health outcomes), and healthy industry to maintain a strong economy.

It is not a novel concept that the pharmaceutical industry is a for-profit industry. However, companies can only make a profit if they produce drugs which patients and society desire. Research and development (R&D) make up the main bulk of the production cost of a new drug, but given the uncertainty in R&D and the need to fund all research including failed drugs and successful ones, it is very difficult for governments to determine the exact cost of bringing a new drug on the market. Furthermore, government imposed price controls decrease the marginal rate of return to industry, which may reduce the incentive for innovation.

Thus regulation of pharmaceutical prices must balance innovation, affordability and cost-effectiveness; pharmaceutical policy must balance the needs of society with the needs of industry.
There are several methods of pricing pharmaceuticals, the three large categories being direct price controls, indirect price controls and free pricing.

**Direct Price controls**
In brief, direct price control refers to the setting of fixed maximum prices, which of course differ across countries, depending on prescribing behaviours, budget limits, patterns of utilization and the importance of the pharmaceutical industry to the national economy. In France pricing is negotiated with a government body for both private and state pricing.

Cost plus and Average pricing are the two main subdivisions of direct price controls.

- **Cost plus** refers to prices being fixed by government based on the cost of production plus a percentage margin.

- **Average pricing** usually refers to a price based on an average of prices from other countries, based on a “basket of therapeutically similar drugs.”

**Indirect Price Controls**
Indirect price controls refer mainly to reference pricing and profit controls.

- **Reference pricing** sets fixed reimbursement limits for products in the therapeutic group or drugs having similar clinical outcomes; all drugs in the group may be reimbursed up to the determined reference price. This type of system is used in Germany, although there are exceptions.

- **Profit controls** strive to make industry responsible for excessive spending on pharmaceuticals. This mechanism does not include generics, and the UK’s PPRS is the perfect example; the PPRS scheme will be examined in more detail in Section 4.4.

**Free Pricing**
This third pricing mechanism includes both on-patent and off-patent drugs. Return on investment and expected demand drive free pricing, with minimal regulation on behalf of the government. The government instead, relies on its purchasing power to keep prices at a reasonable level. In the market of on-patent drugs, there are debates regarding the balance of efficiency and equity, temporary welfare loss and scientific knowledge gained for future innovation.

Generic drugs can undergo free pricing via product differentiation, (new formulations, presentations etc.) thus obtaining an equilibrium price above the marginal cost. If there is no product differentiation, as is often the case drugs are considered adequate substitutes for each other and hence compete on price. This system operates in USA, although health insurance companies will negotiate pricing with pharmaceutical companies and health care providers.

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4.3. Price and profit control for the pharmaceutical industry (PPRS)

The pharmaceutical industry is one of the few industry sectors in the UK that has its profits controlled by the Government. The scheme under which this is controlled is known as the Pharmaceutical Price (Profits) Regulation Scheme or PPRS.

4.3.1. PPRS prior to 2009

As stated in the OFT report in 2007, the NHS spends £11 billion each year on medicines, out of which £8 billion is on branded drugs. PPRS was created around 1959, as an agreement between the UK Health Departments and the pharmaceutical industry, represented by the ABPI, as the main method by which the UK government tried to control prices of branded medicines. The DH and the ABPI negotiate the scheme every five years, aiming to secure value for money for the NHS, while providing incentives to invest in novel drugs for the future. PPRS makes this one of the most highly regulated industry sectors in the UK.

The two main components of the pre-2009 PPRS were profit controls and price controls.

Profit controls imposed a set maximum profit allowed for a company. If this level was exceeded, the company was required to repay this excess amount to the DH. This scheme however, also allowed companies to increase their prices, if their profits fell below a specified minimum.

Price controls allowed free pricing at launch of a new product, but restricted subsequent price increases. There were also one-off price cuts, periodically agreed upon at scheme renegotiations, with some flexibility given to companies in deciding how to deliver savings across different products.

A seven per cent cut was imposed as part of the negotiation of the current PPRS scheme beginning in 2005. Companies are given some flexibility in deciding which products to target in cutting prices, a system known as price modulation.

Manufacturers with NHS sales of £25 million were required to identify products with sales over £500,000 annually and the details of resources used for these products. Companies were allowed a profit of 21% of the return on capital; higher drug prices were allowed to companies that missed their target return on capital by more than 40%. There is also an assessment on Return on Sales with a target of 6%.

The main criticism of the OFT report however, was that neither method, profit control nor price cut, was successful in ensuring that the prices of medicines reflected the health benefits brought to patients. Limited incentives for efficiency and companies overinvesting or artificially inflating asset base, was believed to lead to copycat drugs and to a potential for ‘regulatory capture.’
The OFT was confident that its proposals would closely align the incentives of companies with those of the NHS and allow companies to redistribute the savings towards proof of cost effectiveness.

The OFT stated that the main goal of the new pricing scheme was economic efficiency, which captures the goal of delivering value for money to the NHS and providing the right incentives to companies to invest in drugs for the future; the OFT believes this is central to the interests of patients in both the short and long run.

The OFT report supports cost-effectiveness analysis and as the agency that undertakes such analysis, NICE should play a central role in VBP as establishing the value of a drug is imperative in such a scheme.

4.3.2. OFT market study on PPRS

In September 2005, the OFT launched a study to assess whether the PPRS was meeting its objectives effectively, or whether it was time for reform. In February 2007 it published its market study of the PPRS, stating that that NHS was overpaying for drugs by up to £500 million per year.6 This figure has been keenly questioned and disputed by many. The OFT believed that the PPRS at that time did not offer explicit incentives for companies to invest in the UK, nor did it take account of the therapeutic value of drugs, thus not delivering value for money to the NHS.6 OFT noted that under the scheme drugs with very similar clinical effects could range widely in price, with differences of even 500% noted. The main conclusion was that if prices were not reflective of their value, the NHS was not making the best use of available resources.6

The OFT proposed two options:

Ex post VBP – free pricing at launch of a new product, followed by a series of ex post reviews of the cost effectiveness of the product. The aim of these reviews would be to set a maximum price for the product according to its clinical benefit over time, relative to the most appropriate comparator on the market, including generics.6

Ex ante VBP – a ‘fast track’ ex ante assessment for those products with sufficient data for a cost effectiveness analysis to be conducted at the of launch time. If so, the maximum price reflecting the benefits of the new product, relative to those of a comparator, would be set, along with a decision to reimburse or not. For those products with insufficient data, a ‘risk sharing’ approach could be implemented, under which drugs could be conditionally approved and in time, price could be increased or decreased, depending on the findings of subsequent evidence.6

The report advocates a ‘value-based pricing scheme’ in place of PPRS for all pharmaceutical products not just new ones.

In detail OFT suggested

1. That pharmaceuticals which have come to the end of their patent protection – off-patent, should be priced at a lower level, near generic alternatives, where no research and development has been carried out.
2. Pharmaceuticals with patent protection should be priced according to their ‘incremental clinical benefits’ to patients and broader society.
3. They present two options for value-based pricing the one they prefer has the original price set by negotiation with the Department of Health. Cost-effectiveness is measured regularly and the price reviewed.

The OFT was confident that its proposals would closely align the incentives of companies with those of the NHS and allow companies to redistribute the savings towards proof of cost effectiveness.

However, there have been a number of challenges to the OFT PPRS report. (This will be discussed in further details in section 5.1. entitled: “2020Health.Org Stakeholder Survey on the new PPRS”.)

The value-based pricing scheme model has not been known to operate anywhere in the world. Although the report cites Australia and Sweden as examples, they do not operate such schemes and nor does Japan.

The Australian Model
This has been operating since 1954. The Pharmaceutical Benefits Advisory Committee (PBAC) is an independent expert committee. It evaluates a new medicine for its clinical effectiveness, safety and cost-effectiveness compared with other treatments. If there has no additional benefit from the new medicine over an existing appropriate comparative drug, it may be added to the formulary, so it can be prescribed, but cannot receive a higher price. If it does have additional benefits these are assessed for cost effectiveness against the price. When the PBAC recommends a medicine for inclusion in the Pharmaceutical Benefits Scheme (PBS), the Pharmaceutical Benefits Pricing Authority (PBPA) negotiates reimbursement prices with the supplier. These negotiations take into account not only clinical and cost effectiveness and safety but a number of other criteria. According to Dr Jorge Mestre-Ferrandiz, Senior Economist at OHE, these other criteria include “prescription volumes; the level of activity undertaken by the company in Australia, including new investment, production and R&D (addressed by the Pharmaceutical Industry Investment Programme); prices of the drug in reasonably comparable overseas countries; and other relevant factors which the applicant company may wish the PBPA to consider; and any directions of the Minister”. This is not therefore a value-based pricing system as outlined by OFT.53

The Swedish Model
“Sweden introduced a new pricing and reimbursement scheme in 2002. Its main features are the use of cost-effectiveness analysis for determining the reimbursement status of new pharmaceuticals and mandatory substitution of the lowest-cost generic alternative. The use of cost-effectiveness analysis in reimbursement decisions helps to relate the reimbursement price paid to the social value of the product, but does not necessarily result in the lowest possible price.”54

There is no formal price regulation in Sweden. However, if a company wishes to have its medicine reimbursed by Pharmaceutical Benefits Board (LFN) it must be cost-effective. This reimbursement system exists for outpatient prescription drugs and some over-the-counter (OTC) medicines, but not for hospital medicine prices. These are negotiated directly with the County Councils, which are responsible for hospitals. Again this is also not a value-based pricing system as described by OFT.54
There is also some criticism of the examples given of cost savings by using generics or older drugs instead of newer branded ones. The cost savings were questioned by Towse, specifically the savings of £350 million on simvastin, a statin for reducing cholesterol levels.35

OFT reported “For one drug alone we estimate that the use of more value-reflective prices could potentially have saved £350 million in that year.” Towse’s response – “Yet the OFT knew at the time of writing that the savings from substituting use of generic simvastin in the place of Lipitor were much lower.”

There were no GPs and only one hospital consultant on the panel. GPs are aware of patient feedback on the acceptability, effectiveness and side effects of drugs, directly from their patients. Patient compliance was mentioned but not analysed or costed as part of the benefit of using newer drugs with potentially less side effects. The savings mentioned are simplistic and unlikely to be realised.

4.3.2. 2009 PPRS

In August 2007, the Government published its interim response to the OFT report, welcoming it and promising to take into account its recommendations: delivering value for money, aiding the uptake of new medicines, rewarding and promoting innovation, while supporting industry by providing stability, sustainability and predictability.30

In their June 2009 ‘Final Government response to recommendations aimed at Government contained in the OFT report “The Pharmaceutical Price Regulation Scheme”, the Department for Business Innovation & Skills stated that the Government believed that value was ‘already reflected to a certain level by the manner in

The pricing levels in the OFT report showing the UK at higher prices than other European countries are questionable, as list prices are used, which does not take into account the many discounts, patient access schemes, tenders and even reverse e-auctions which drive pharmaceutical prices down.

More recent price comparisons show that UK prices are lower than other European countries. (ABPI) Please see Figure 3 below. Prices have been falling in the UK relative to other leading economies for the last few years. Please see graphs from previous years in Appendix 2

In the first quarter of 2006 UK prices ranked 5th, but this fell in quarter 1 2008 to 9th and again early in 2009 to 12th.

The Japanese model is very complex and currently being changed. The current version is described briefly here, not the new one, which is due for introduction in 2010.

The current drug price standards are based on the ‘The Standards for Calculating NHI Drug Prices’ developed by the Central Social Insurance Medical Council on February 13, 2008.

A new drug price is compared with existing similar drug prices. Where a new medicine is shown to have benefits over existing drugs a premium is applied. There are four premiums for –

- Innovation (70-120%) – improved therapy, greater safety or efficacy, new mode of action
- Usefulness (35-60%) – Improved therapy, high level of safety or efficacy
- Marketability (5-20%) – orphan drugs etc for rare diseases
- Paediatrics (5-20%) – specific indications, presentations and administration for children

If a new medicine is found to have low innovation with similar drugs already on the market is will be priced at the lowest of the similar drugs.

If there is no comparator the costs are taken into account.

Pricing of the same medicine in USA, UK, Germany and France is also taken into account.

This is not the value-based pricing system as described by OFT. (With thanks to ABPI)
which the NHS operated and purchased drugs. Furthermore, it was stated that NICE played and plays a crucial role in this process, for through its HTA, it indicates ‘under what circumstances the use of a particular drug is both clinically and cost-effective’ (p. 5).

Acknowledging the need to reflect value in the prices of medicines, the 2009 PPRS describes the link between pricing and value to patients in stating the interplay between the PPRS and NICE. The 2009 PPRS introduced a more value-based approach to pricing of pharmaceuticals via two main mechanisms: Flexible Pricing and Patient Access Schemes.

Under Flexible Pricing, drug prices can be increased or decreased as further evidence becomes available that may change the effective value to patients in the NHS. If the new evidence supports increased benefits for existing indications for a product, the price can be increased to a maximum of 30% of the list price. If new indications arise, then the manufacturer can demand a once-only increase in price, but must keep the old price when the medicine is prescribed for the original indications. The mechanisms for this price increase have yet to be finalised.

Patient Access Schemes are aimed at improving patient access to drugs which were not initially found to be cost effective by NICE, possibly because there is insufficient evidence. Arrangements are made between companies and the DH, allowing manufacturers to offer discounts or rebates to reduce the effective cost of a drug to the NHS. The two main schemes agreed upon by the DH and the ABPI are: financially-based schemes and outcome-based schemes.

The OFT market study on the PPRS also stated that NICE’s HTA should include benefits to the patient, but also others around the patient, giving carers as an example. The Government has commissioned an independent study by the University of York into the potential effects of extending NICE’s economic perspective. This study is due to be available later this year.

To assist the uptake of new medicines and in order to promote and reward innovation, the 2009 PPRS includes an ‘Uptake and Innovation package’, which further supports the OFT’s recommendation that ‘prices should reflect the value of medicines to patients and society’, and that ‘NICE has an important role to play in determining whether the manufacturer’s price represents value for money’.

It should also be appreciated that most companies will review pricing of their products annually to take account of inflation, currency fluctuations, changes in raw material prices and the market etc. Under the PPRS pharmaceutical companies cannot change pricing unless they apply to DH to do so. It is often the case that the list price does not change from the price at launch and the companies have to absorb the increased costs.

4.5. NICE - Cost Effectiveness Analyses

It is useful to remind ourselves of the purpose of NICE, as its aims are often misreported.

The National Institute for Clinical Excellence (NICE) was first introduced to us in the Government white paper ‘The New NHS – Modern, Dependable’ in December 97. Its purpose was “to give a strong lead on clinical and cost effectiveness, drawing new guidelines and ensuring they reach all parts of the health service”… “The new NHS will have quality at its heart” and “quality and efficiency”… “will go hand in hand”.

The appraisal by NICE of both new and existing technologies encompasses:

- clinical effectiveness
- cost effectiveness
- wider NHS implications

Before NICE, new treatments were reviewed locally e.g. by drugs and therapeutics committees. This led to differences in availability of treatments - “the post code lottery” - but did give local clinicians much more flexibility.

It is much more efficient to have a central review body that has built up considerable expertise, but local flexibility for individual patient needs suffers as a result. NICE has the unenviable task of saying ‘no’ to patient treatments and taking the blame for unpopular decisions, once shouldered by the government.

However, the introduction of NICE has increased the costs to pharmaceutical and medical device companies considerably, as they have to provide cost-effectiveness evidence for NICE, as well as safety and efficacy evidence to MHRA. This necessitates additional expensive economic trials. This is not a requirement in other developed countries.

NICE does produce some very useful guidelines, which do promote clinical excellence and would improve treatment outcomes and patient health. However, it is not mandatory for PCTs (Primary Care Trusts) to adopt these Clinical Guidelines and they are all too often ignored. (They are, however, required to implement Technology Appraisal recommendations, although there is evidence to show that this is not always the case.) In some people’s eyes it has unfortunately become a negative force; if a NICE review is negative it will stop the new drug or product being used in the NHS; if positive it may make little difference to its adoption and usage.

NICE has been criticised by the Health Select Committee and a number of patient groups, companies, doctors and other stakeholders for its assessment methodology. Its remit from the Government is only to consider costs to the NHS and social services; not those to patients, their families and carers, their employers or the economy as a whole, or their ‘values’. There has been criticism and even a court case about the transparency with which NICE conducts appraisals. It is possible that many findings on ‘value’ and therefore pricing could be challenged in court.

In England and Wales NICE is responsible for developing guidance on effective public health practice, on interventional procedures and clinical guidance. Of course it also undertakes medical technology appraisals covering medical devices, diagnostic techniques, surgical procedures, health promotion activities and medicines referred to it by the Secretary of State. (Scotland has its own body with different methodologies.)

One of the main rationales for the creation of such a body is to secure value for money in the use of the limited resources of the NHS. It does this within its Health Technology Appraisal process (HTA), via cost effectiveness analysis. Cost effectiveness analysis (CEA) allows the comparison between clinical benefits and costs of various health interventions, with the aim of achieving better and more rational allocation of resources.
The topic for appraisal by NICE comes from the Department of Health, medical and public health professionals, patients, carers, NICE itself and from the National Horizon Scanning Centre, but final approval from the DH is necessary.42,43

The team of reviewers comprises experts in the field to be assessed, public sector representatives, patients and carer representatives.42

The traditional appraisal process at NICE is called the Multiple Technology Assessment (MTA). Under this process, NICE commissions independent academic centres to review the evidence on the topic, to develop a cost effectiveness model and prepare the final report. An appraisal committee is an independent group composed of NHS health professionals and other individuals familiar with the topic at hand.42 This committee seeks the views of organisations representing patients, carers, health professionals, the government and manufacturers.

The Single Technology Assessment process (STA) is a process which is 6-15 months shorter than the MTA, for it reviews each single submission, without developing a separate economic model.46

As the OFT report6 lists them, the stakeholders involved and their roles are:
- National patient and carer groups submit evidence and comments
- Health professional bodies recommend those to be consulted
- Research groups comment on the appraisal documents
- Manufacturer comment on the appraisal documents and may appeal the final decision
- Members of the public and other health professionals feedback post publication of final decision

In brief, HTA assesses the relative cost effectiveness of a new health technology by estimating the incremental cost and health benefits, versus those of the comparator. A comparator health technology is the most clinically or cost effective available treatment or the most widely used treatment, at the time, on the market.6

The costs used in an appraisal process include the public list price. Sensitivity analyses are applied to assess variation.42 If no list price is available, then the price submitted by the manufacturer is used, as long as it is publicly available.52 Value added tax is excluded from the economic model, but considered for the budget impact.42

NICE recognises that some health technologies can impact costs or savings on other governmental departments.42 In such situations, NICE considers these costs or savings as long as they have been agreed with the DH.32 NICE however, does not include these costs in the incremental cost effectiveness ratio (ICER) calculations. An annual discount rate of 3.5% is applied to both costs and benefits.42

The benefits measured are health benefits and adverse effects to patients and/or their carers.42

A popular measure of the therapeutic benefits delivered by a health technology is the QALY - quality adjusted life years. The QALY is a measurement tool for economic evaluation, which takes into consideration the quantity and quality of life, as a result of a medical intervention. A year in perfect health is equal to 1 and death is equal to 0 QALYs. Utility weights are applied to different health statuses, derived from surveys, such as the EQ-5D and the Time Trade-off questionnaires, of patients’ preferences over different rages of possible health states. The QALY forms the basis of the ICER, which is the main tool used for determining the value for money of a new treatment relative to an appropriate comparator.

EQ-5D is used to measure quality of life and Jeffrey A. Johnson and A. Simon Pickard in their paper comparing EQ-5D and SF-12 (another assessment model) set out the system used, which “consists of five dimensions -
- Mobility
- Self-Care
- Usual Activities,
- Pain/Discomfort
- Anxiety/Depression

with three levels each (no problem, some problems, extreme problems), thus defining (35) distinct health states. The EQ-5D also includes a visual analogue scale (VAS) as a means of valuing the health state of the respondent independent of the descriptive system. The endpoints of the VAS are labelled ‘best imaginable health state’ and ‘worst imaginable health state’, anchored at 100 and 0, respectively. Respondents are asked to indicate how they rate their health by drawing a line from an anchor box to the point on the VAS which best represents their own health that day. While it is considered an index measure, responses to the EQ-5D can be presented variously as a derived index score, a VAS score, and a profile of responses to the dimensions.”

It has been criticised by some patient groups in particular for not including other health issues such as incontinence, which is obviously an important consideration for those afflicted. NICE does take other health issues into account, but some feel that many other health issues need to be included formally and properly assessed with complete transparency.

NHS and social service costs are included, but not wider costs to patients, their families (unless carers) society, the economy, employers etc.

Some might feel that basing such important decisions on a cost/QALY calculation alone does not accurately and adequately reflect views and values of society, NICE created a Citizens Council. This comprises 30 individuals from all population groups, who meet twice per year to discuss specific ethical issues brought forth by NICE, as representatives of society and its value judgements.6,42 Furthermore, NICE develops principles that form the foundation of the social value judgements, stating that priority should not be given based on demographic characteristics, except when it is clearly evident that such characteristics are clinically relevant.42

When there is a trade-off between the costs of a new product and its benefits, NICE bases its recommendation on the ICER. There is no official threshold that a new health technology is compared against, but NICE usually recommends new products that fall under a threshold of £20,000. A technology with an ICER greater than £20,000 necessitates an investigation of the degree of uncertainty surrounding its calculation, the distinguishing features of the illness being targeted and its patient population; and a more detailed look at the wider societal costs and benefits, and the level of innovation.6,44
Some like Towe argue that there is no scientific basis for the threshold.35 Indeed if it is compared with the NHS tariff for specialist hospital treatments there are a number of procedures which cost over 4 times the threshold value. The threshold applies to pharmaceuticals, medical technologies/devices and other treatments that go through the NICE HTA process for clinical and cost effectiveness. The same thresholds do not apply to NHS procedures. This would appear to be an anomaly.

The cost per QALY estimate varies very widely for an individual technology assessment. The output is not one definitive estimate, but a range, which can vary widely e.g. from 5 to 300 for one technology. It would be very difficult to set a price based on a very wide range of costs per QALY for one technology.

4.6 The Kennedy Report

Following Sir David Cooksey’s Review and Refresh of Bioscience 2015, Sir Ian Kennedy was asked by Sir Michael Rawlins, the Chairman of NICE, to undertake a ‘short study valuing innovation’.

The report begins by explaining the need for health economics in healthcare, and clarifying that NICE has ‘no role in setting or even negotiating the price that a manufacturer may propose. (NICE) simply works on the basis of what is put before it’9. The first recommendation centres on NICE and its duty to be more active in developing more strategies to achieve its goals. It states that NICE does accept debates and challenges, but does not make the public aware of this. He supports NICE’s Social Value Judgements document as being a very good idea, but criticises it for being too abstract, not practical, applicable, nor very realistic.

The report then goes on to comment on the pharmaceutical industry, which in his opinion acts for the individual patient, whereas NICE tries to provide for society as a whole. It is to be stated that the healthcare market is not a perfect market. Kennedy recognises that there are asymmetries of information in the healthcare market, and he sees NICE as the link between provider and consumer, one of its purposes being to ease this asymmetry. He acknowledges that indeed, we now live in a global society with a global market, and he proposes that NICE should work with industry on how costs of R&D are distributed in the global market.

Recommendations 3, 4, 5 and 6 are of great relevance to our study:

- **Recommendation 3** - that NICE appraisals should continue to be based on the ICER/QALY approach etc.
- **Recommendation 4** - NICE should consult all relevant parties and draw up a list of those health related benefits to be taken into account in its appraisals
- **Recommendation 5** - NICE should begin to adjust its evaluation of QALYs in advance of any research findings on methodology, in consultation with relevant parties, so as to begin to take account of relevant health-related benefits once identified and agreed

Sir Ian Kennedy recommends that the ICER/QALY should continue to be the central point of HTA, with three important matters that need to be addressed: benefits, their weights and the threshold. Kennedy puts forth examples of health benefits related to him via submissions from stakeholders, such as patient dignity, independence, side effects, inpatient versus outpatient treatment, different modes of administration of the treatment, and so on. He proposes that NICE consult relevant parties and agree on the benefits for inclusion with timely reviews. He challenges NICE, stating that it could be argued that these benefits may already be factored into the ICER/QALY calculations, but this is not happening in a transparent manner. He calls for research to determine whether the QALY captures such health benefits and whether they are applied and weighed appropriately.

**Recommendation 6**, (The presence of benefits of the sort referred to (mode of administration, site of treatment, reduction in unwanted side-effects etc.) should not result in an increase in the threshold used by NICE) brings us a step back. The definition of the threshold from Culver and colleagues is ‘in the presence of a fixed budget, which cannot be changed by the decision-maker, the cost effectiveness threshold is not a measure of willingness to pay for health. It is an estimate of the opportunity cost (health forgone by others) of substituting a new technology into the portfolio of technologies provided’9 (p.26). Professor Sir Ian Kennedy agrees with this definition and states that the added social benefits mentioned above should not form the basis of a higher threshold. However, if such benefits are deemed as ‘value’ by society, such a technology may fail to be recommended by NICE, if its ICER/QALY is higher than the standard threshold of £20,000 - £30,000.

**Recommendation 7** (Social benefits ... should not currently be taken account of by NICE in its appraisals, but NICE should commission or participate in research to determine whether such benefits could form part of NICE’s approach) is highly important, especially after having reviewed the findings of our stakeholder interviews. Currently, NICE factors in only costs and benefits to the NHS (and personal social services). Sir Ian Kennedy recognises that there would be privileges for worker and associated tax revenue if social benefits to the economy, such as whether a product allows carers to lead a more independent life, allows patients to return to work and hence increase tax revenue, and reduced social costs, were factored in. However, he points out that it would be very difficult to calculate such benefits, but does encourage NICE to commission or to participate in research to determine methods of how to incorporate such social benefits into the appraisal process. He also states that if such benefits are to be considered, they should be considered with the ICER/QALY analysis at its centre.

Professor Sir Ian Kennedy also raises the issue of the limitations of the questionnaires used to elicit the value of a QALY. He informs us that work is in motion, to address these methodological issues and the calculation of the cost-effectiveness threshold, via a grant from the Medical Research Council, and NICE’s joint work with the EuroQol group to identify possible ways of improving the QA-5D tool.

Later the report tackles issues of innovation in the world of healthcare technology. The term innovation is used implying ‘different ways of doing things, which bring improved outcomes’, as suggested by Sir David Cooksey. If the claim is for an ‘innovative’ product to receive special treatment in the form of a higher price or a higher threshold, then that product must be new or different and more effective than the products already on the market. He does not agree that NICE should have to incentivise manufacturers in their pursuit of innovation, more than it already does.

He does however suggest that new products, which constitute an improvement on existing products and which offer more benefits, ’a step-change in terms of outcomes for patients’ as he describes it, should be recognised for their added social value of innovation and hence a variation of NICE’s appraisal process would be justified.

In recommendation 15, (NICE should establish a mechanism whereby pharma can signal as early as possible that a product may constitute an innovation … ) Professor Sir Ian Kennedy sets out the mechanisms for responding to innovation. As part of this, he recommends that there should be a higher QALY threshold permitted in the case of innovation for 3 to 5 years. Since he sees the NHS budget as being fixed, the threshold for ‘non-innovative’ drugs would be higher. In this context, he was somewhat dismissive of the ‘semi-courageous’ £25 million Innovation Pass which ‘has to be watched’. He feels this is too small an amount. There is a risk that the initiative could be seen as a ‘Trojan Horse’9 (p. 35) and hence has to be operated by NICE and must not be absorbed into NHS budgets. He also recommends that NICE should establish a method by which manufacturers reimburse/compensate it, if a product does not eventually meet initial expectations.
Furthermore, the report supports cost sharing/joint funding, a need first recognised by Sir David Cooksey. He recognises that continued research on the surveillance of a product is necessary and expensive. He looks at the US as an example of the $4 billion federal taxpayer money spent by the NIH (National Institute for Health), on supporting clinical trials. He sees this type of contribution as bringing health and financial benefits both to the NHS and to the industry. He does however, not consider this a matter for NICE, but rather as a collaboration in research and collection of data between the DH on behalf of the NHS and industry; although he makes note of ‘tensions in the Department of Health over funding’ (pg 46, footnote 70), hence suggesting that NICE work with the Office of Life Science ‘to ensure that the proposals of the office relating to research and data collection are acted upon promptly’. (P. 47) and suggests that the R&D costs of rare diseases could be subsidised through involvement of the public purse, or via an increase in the ICER/QALY threshold.

The report concludes with a brief overview of the status of diagnostic tools, devices and psychological therapies. This is an area which our interviews have found dissatisfaction with, on behalf of the manufacturer. Diagnostic tools and other medical devices have important differences compared with pharmaceutical manufacturers, thus Kennedy is happy with NICE’s decision to establish the Medical Technology Advisory Committee to evaluate these types of products. With regard to psychological therapies, he recommends that NICE work especially with Academic Health Science Centres to ensure firm grounds for research and gathering of evidence in this domain.

Chapter Five

5. Value-based pricing – What is it? – Assessment and implications

There is no accepted definition of ‘value-based pricing’. One might argue that prices set by ‘customer value’ are value-based prices and this is not a new concept, but has been with us for some time in many sector, particularly the sophisticated markets such as luxury goods, electronics, fashion etc.

Looking at pricing research in the marketing and business sector the consensus seems to be the ‘value-based pricing’ is pricing a product or service at the level at which the customer and consumer ‘value’ the product relative to others. This would be taking into account the costs and benefits as shown in Table 1.23-31

OFT market study on the PPRS argues that VBP provides value for money for the NHS, while giving manufacturers incentives to invest in future innovative drugs, hence making the system more efficient in the short and long run.

However, there still does not seem to be a clear definition of what ‘value based pricing’ is in healthcare, neither in the OFT report nor the 2009 PPRS. Therefore some pertinent questions are:

- What is Value Based Pricing?
- How does Risk sharing fit in?
- Does VBP affect domestic R&D and/or investment?
- What would its effects on an international scale be?
- How does VBP impact innovation?
- Do cost effectiveness analysis and the QALY effectively measure and capture value and value for money?
- What about cancers, other terminal illnesses and orphan drugs?
What is value-based pricing (VBP)?
During the interviews, academics and health economists defined VBP as the price that reflects enhancement of welfare; the maximum price afforded by the NHS. Government and the NHS Confederation felt it was important to consider the government’s budget. (This is in accord with Claxton’s definition of VBP as the price ‘that ensures that the expected health benefits exceed the health predicted to be displaced elsewhere in the NHS, due to their additional cost’ (p. 545). In this case, the main principle then becomes the outcome of a comparison of the ICER between the new technology and the comparator, with a threshold for cost-effectiveness.

Given a fixed budget, it seems that VBP could be considered as a measure of CEA. In this case however, the definition of ‘value’ and ‘value to whom’ must be clearly stated. Both the stakeholders interviewed and the pertinent literature considered this the most important challenge ahead.

Clinicians described ‘value’ as a subjective term, not quantifiable, but indicative of wider measures of health gain. Suggestions put forth were that in addition to benefits incurred by patients directly, the non-patient benefits should also be considered. This ties in with the OFT report, which states that benefits to carers count towards the determination of value.

Most stakeholders believed that the principle of VBP rested on outcomes to the individual and benefits to society. Industry representatives felt that VBP should take into account innovation and benefits to the patient via extension and/or improvement of quality of life, and benefits to society in terms of benefits to carers and ability of patients and carers to return to work.

Moreover, the majority of the other stakeholders also agreed that ‘value’ is a wide concept, but stated that only value to the NHS and social services costs are considered in a HTA. The NHS’s goal is enhancement of the general health state of the population. In an ideal world each individual’s needs and desires would be considered equally. Manufacturers target their drugs to specific illnesses and the specific individuals suffering from them. Government however, must concern itself with the good of the greater. Unfortunately, given the reality of budget limitations, the greater good comes at a cost to the few.

Medical device manufacturers showed concern over the fact that it is more difficult for devices to prove a specific health outcome or even a direct benefit.

Overall, there was a sense of a need for public debate on value. Most recognised that as proposed for the UK, a VBP scheme is yet to be observed in other countries. Although some stakeholders gave Germany as an example, the OFT report uses the German system as an example of ex post ‘pricing relative to substitute’ (p. 71). That reimbursement technique is similar to a therapeutic reference pricing system. However, it must be kept in mind that with a VBP model that the OFT proposed, a new drug must prove more benefit or lower cost, and a price would be set based on the benefits it brings, rather than clustered with similar pharmacological or therapeutic substitutes.

The OFT also proposed that generics, if available, should be considered as comparators. Some companies have argued that on-patent drugs should not be compared against generics, but the OFT was clear in stating that given the limited resources of the NHS, it cannot afford to ignore such comparators. When interviewed, the OFT affirmed that its role was to recommend, but that the detailed definition and process of defining and calculating value was up to NICE or the Department of Health (DH) to determine.

5.1. 2020health.Org Stakeholder Survey on the New PPRS

We interviewed 35 stakeholders. To ensure that we gained the perspective of all parties and groups involved and affected. A full list is shown below. We would like to thank all respondents for taking part, namely:

- **Patient groups:**
  - Cancer Research UK
  - National Voices
  - National Rheumatoid Arthritis Society

- **Medical Technology Group:**
  - Chair and Heart Research UK – National Director
  - Boston Scientific – Director of Policy & Government Affairs & Health Economist
  - Medtronic – Government Affairs

- **Government and NHS:**
  - NICE – Chief Executive
  - DH - Head of Clinical and Cost Effectiveness Medicines, Pharmacy and Industry Group
  - NHS Confederation - Director of Policy & Communications
  - OFT – Author of PPRS Report 2007
  - National Social Marketing Centre – Director

- **Royal Colleges of Medicine:**
  - Royal College of General Practitioners – Vice Chairman
  - Royal College of Surgeons – Lead on NICE
  - Royal College of Physicians – Vice President (Academic)

- **Academic Health Economists, Policy Makers:**
  - Prof. Julian Le Grand – Professor of Social Policy - LSE
  - Prof. Karl Claxton – Professor of Economics – University of York
  - Prof. of Health Economics - LSE
  - Ms Valerie Amies

- **Partners:**
  - ABPI – Commercial Director
  - ABHI
  - Office of Health Economics- Deputy Director
  - IMS Health – Engagement Manager – Health Economics
  - Medical Marketing Group CIM
    - Richard Featherstone
    - Stewart Stanley

- **Healthcare & Vaccine Companies:**
  - GE - Reimbursement Leader – EMEA, Health Economist
  - Sanofi Pasteur MSD – Deputy Managing Director

- **Sponsors:**
  - Boehringer Ingelheim
  - Genzyme
  - Lilly
  - Pfizer
  - sanofi-aventis
  - Shire
R&D investment and innovation - multiple reviews
Both, the pertinent literature and the stakeholders interviewed saw VBP’s potential to go either way both, to incentivise and to inhibit innovation. In his recently released report on valuing innovation, Sir Kennedy expressed his opinion that incremental innovation should be recognised for their added social value of innovation and hence a variation of NICE’s appraisal process would be justified. If a new drug whose sole distinguishing characteristic from others of its class is a different method of administration, but which could positively impact on a patient’s quality of life, it should be rewarded and not grouped within a therapeutic reference class. There were however, concerns about how such incremental innovation would be measured. An example of a new insulin analogue was given, which made no difference in clinical outcome or cost, but industry believed it to be innovative in that with this new version, the patient would not have to wait thirty minutes after meals. This ‘subtle’ innovation might be important and valuable to patients and their carers.

Paying attention to the parameters used to measure and define value is imperative if VBP is to be described as a scheme designed to drive innovation. Most interviewees agreed with this. Supporters of the OFT’s proposal believe that VBP rewards incremental innovation and benefit, since marginal improvements in treatment would receive higher payments. Most interviewees judged that VBP would ultimately help companies that prioritised on and invested in products with most innovative potential.

The Flexible Pricing scheme under the 2009 PPRS allows prices to be increased or decreased, as further evidence becomes available and is collected, similar to an ex post manner. Some stakeholders viewed this as a good means to encourage high quality evidence on a product. Most industry representatives however, noted that since innovation under the new guidelines requires new evidence, this adds an element of risk for companies. Unless the value of developing the new technology and the costs of ongoing evidence collection is taken into account, companies may shy away from uncertainties, and hence not develop truly novel and innovative medicines. The literature shared in this concern.

Patient access schemes
Patient Access Schemes come into play at this point, for if the evidence is not available at launch, then a second review would be undertaken. However, manufacturers stated that continued research on the surveillance of a product is expensive. The Kennedy report looks at the US as an example of the $4 billion federal taxpayer money spent by the National Institutes of Health on supporting clinical trials. For the UK, he sees this type of contribution as bringing health and financial benefits both to the NHS and to industry. He does however, not consider this a matter for NICE, but rather as a collaboration in research and collection of data between the DH on behalf of the NHS and industry.

If companies are expected to fund ongoing research and data collection, this may act as an incentive to launch the product at maximum possible price, in order to maximise their returns on investment. Although a price increase is possible in theory, most industry stakeholders were sceptical and believed that with multiple reviews, the price would most likely be lowered. NICE always indicate that it is only responsible for the evaluation of the product, whereas price negotiations take place with the DH. However, it seems that if VBP as proposed by the OFT were to be fully adopted in the UK, then NICE might be viewed as “the government’s price negotiator for drugs” (p, S10).

International price benchmarking
Industry pointed out that research and development (R&D) is performed on a global level. The UK is used for international price comparisons for other countries (price benchmarking) such as Japan [previously mentioned], Switzerland etc. A low price in the UK could significantly affect pricing and profitability in other countries and globally. They did not necessarily envisage a change in the dynamics on an international scale, but they felt that pricing based on value via multiple reviews, would result in a significant decrease in prices, a factor which would drive manufacturers away from launching in the UK, or certainly from launching early.

Despite the worries of the industry, health economists and academics did not believe that the international implications of a VBP scheme in the UK would be as large as envisioned. They pointed out that VBP could encourage manufacturers to think more carefully about the type of evidence and the type of products they invest in, thus bringing forth innovative products.

The pertinent literature dismisses this fear. Legally, the PPRS is not able to differentiate between companies with R&D in the UK or abroad, and the choice of location is influenced by local costs, local taxes and degree of public investment in research. Furthermore, development, production and marketing of a product can take place in different locations and be available worldwide. If ‘VBP is not about reducing overall expenditure on drugs, but making best use of expenditure’ (p. 986), then such fears can be dismissed.

Moreover, Towsé considers the relationship between price and volume. In theory, a drug recommended for a wide variety of conditions will have many consumers and so can afford to obtain a lower price, whereas a drug targeting or restricted to a specific sub-group of patients could command higher prices. He ponders whether the balance in the healthcare market rests on price, volume or a combination. Towsé believes that the OFT assumes adjustments are made through price alone, for if prices do not reflect value at certain volumes, it assumes price rather than volumes to be wrong. When this was mentioned during the interview, the OFT pointed out that “both price and volume can be adjusted but the end position must be something where the price is still reflective of the value of the product”.

Regarding sub-group analysis specifically, NICE often analyses effects and costs by patient sub-groups and confines its recommendation to those for whom the respective technology is cost-effective. This is fortunate to the manufacturers, for instead of completely rejecting a new drug, NICE accepts it for certain groups.

Overall however, the interviewees suggested that a constructive discussion between patients, policy makers and industry is necessary and due.

Cost effectiveness assessments and QALYs
Government and the NHS Confederation supported CEA as the tool to determine value and value for money, seeing the QALY as a common currency of comparison across different groups of patients. However, other stakeholders described CEA as unable to distinguish between therapeutically similar drugs. Several industry stakeholders described CEA as a ‘blunt guide’ that ‘provides a narrow mathematical formula’, stating that NICE’s economic perspective is defined by the DH, ignoring any benefit beyond that to the patient.

The general opinion was that current CEA fails to capture effects of patient travel time and convenience, benefits of patients and carers returning to work, paying taxes and reducing the need for social care. There seems to be a widely held belief that none of these externalities are properly measured by the QALY, or by the Markov Model when modelling CEA.

Recommendations for improving CEA’s ability to capture value and value for money were to broaden its
Furthermore in his report, Sir Ian Kennedy⁹ praised NICE’s Social Values Judgements document, but criticised it for being too abstract, not practical, not applicable, nor very realistic.

Moreover, although the QALY is generally agreed upon as a fundamental concept, there is still debate about whether a QALY is a QALY or a QALY. If the QALY is a theoretical measure, then QALYs are equivalent and interchangeable, despite age and health status. This is not realistic however, for we, as a society, already value certain aspects of life and healthcare more than others.⁴³

With regards to diagnostics, stakeholders felt the QALY not to be so relevant; a CT scan or an MRI does not produce a clear outcome. In the case of vaccines, the use of the QALY poses problems as well, for issues such as herd immunity and discounting the rate of the impact, skew the results.

Many stakeholders pointed out that the economic empirical evidence shows that QALYs are different across individuals, hence different attributes and characteristics should be weighed differently.

Furthermore, there is a lot of uncertainty surrounding it; it is dependent on and relies on information. Some stakeholders did not see issues with the QALY per se, but with the mechanisms used to elicit its value. The EQ-5D and the Time trade-off questionnaires were believed to not be sensitive enough, or is the data that goes into them. Others found many flaws with the EQ-5D, but recognised that there is no other better tool. It is reassuring however, that the methodological limitations of the QALY have been recognised and are being addressed.⁴⁴

It was suggested to continue with the current methodology, but increase the liberty of decision making, especially in areas not fully covered by the EQ-5D. Kennedy⁹ also examines this issue, but informs us that work is in motion to address these matters along with the calculation of the cost-effectiveness threshold to be addressed in the next section, via a grant from the Medical Research Council, and NICE’s joint work with the EuroQol group to identify possible ways of improving the EQ-5D tool.

Most recognised that no one is truly an advocate of the QALY, for it represents a comparison and trade-off of healthcare. However, given a limited budget, there seems to be no other choice; some stakeholders believe that for every patient who is treated, another is not.

Until we solve the problem of a limited budget, so that NICE will no longer be viewed as a rationing body, the QALY seems to be here to stay. What we can improve on, are the uses of the QALY and its elicitation methodology. The elicitation questionnaires are community based and help assign a value to the QALY. As one stakeholder noted, independent patient preferences would provide better quality data, the ideal information, in fact. To gather and analyse all this data though, would be costly.

For now, as a policy maker stated, we cannot take an average of the data; we need to make sure the sample is representative, thus we must be able to defend the sample of the population used for the creation of the QALY.

The overall feeling was that the QALY is ‘the best we’ve got thus far’, and we should focus on improving its elicitation, general technique and its use.

Threshold

An equally important factor in HTA is the threshold. Currently, NICE recommends products falling below a cost/QALY threshold of £20,000.⁴⁷ For medicines with an ICER greater than £20,000, NICE requires an investigation into the uncertainty of the ICER, the particular features of the condition and population receiving the technology and a more detailed look at the wider societal costs and benefits and degree of innovation.⁴⁵ This has raised debate of whether there should even be a threshold, and if so, how should it be determined? Given reimbursement decisions and an assessment of value for money, then a threshold is necessary.⁴⁵ Therefore, a proper definition of this threshold is crucial, especially within a context of VBP.³¹ The manner in which the threshold is defined, whether by a political process, by a ‘socially legitimate process’, or based on negotiations with industry, can affect the HTA process, the health outcomes and prices of the new products.³¹ A year later, Claxton et al.³² again stress the importance that NICE’s threshold should be based on scientific evidence. Setting an explicit threshold would add transparency and consistency to the decision-making process.³³ On the other hand, if the opportunity cost of a new product is to be considered, as Kennedy⁹ believes, and then based on such a definition, he would deny a higher threshold on the basis of added social benefits such as mode of administration, site of treatment, reduction in unwanted side-effects.

In order to implement a VBP scheme as per the OFT, the worth of a QALY must be known, and a threshold must be present. However, most stakeholders believed the current threshold to be an arbitrary one, not determined in a scientific or democratic manner, but rather set by economic theorists. It was pointed out that refusing prices above threshold, for fear to take away from other treatments, assumes that everything in healthcare has a set cost/QALY. It is therefore quite clear that more research is necessary to try to elicit the prices of the population and improve the rationing process of healthcare.

Many stakeholders asked whether all treatments should have the same cost-effectiveness threshold, whether all diseases merit the same level of value. Medical marketers thought that there should be different cut-offs related to age, duration of benefit and relative contribution to the quality of life.

The January 2008 report by the House of Commons health select committee³⁶ investigated the threshold used by NICE in its appraisal process. The committee found that the range had no scientific basis, did not consider costs to carers and social services, nor did it relate to the NHS budget. Further research was recommended.³⁶
Cancers; Rare Diseases; End of Life

Most stakeholders were split in their opinions on the ‘value’ of these conditions. Some believed that such conditions should be treated differently. Several industry stakeholders expressed a desire to see medical technology aimed at marginal groups be potentially excluded from a regular pathway. An example was given: a new drug providing benefit to 30 percent of the individuals suffering from a lethal illness, but not cost-effective to others. How do we deny people hope? Three months to a cancer patient may be life changing, while insignificant and taken for granted to the healthy.

Another stakeholder also wondered whether it would be ethical to deny treatment to a small number of patients just because they happen to suffer from a rare disease? As noted, the point of the matter is that given the larger demand, the supply side shows that there is more technology available for the more common illnesses.

Others did not see reasons for leniency, noting that placing more value on certain drugs would be inequitable. McCabe et al.49 point out that putting more emphasis on rare diseases takes away from the common ones, which could be just as serious. However, the argument for orphan drugs has been put forth: despite the lack of cost-effectiveness of many of these medicines, it would be unfair to patients suffering from a rare disease not to be treated; society may still prefer to offer care to these people.46 NICE seems to be starting to consider the differences in social values for different groups of patients, especially those with rare diseases. In July 2009, the Innovation Pass37 was introduced; an action within the Government’s Life Sciences Blueprint, and administered by NICE. Set to start its pilot year in 2010, the Innovation Pass37 will allow highly innovative drugs, targeted at small patient populations with rare diseases, to be available to the NHS for three years. NICE will consult with stakeholders at the end of this year and set up a criteria for granting this Innovation Pass.39

The Innovation Pass seems to come as a complement to the End of Life Medicines Guidance48 issued last year, aimed specifically at treatments used to extend life of terminally ill patients, and with an ICER greater than £30,000. At the announcement last year, Andrew Dillon, CEO of NICE, was quoted saying that “the Institute is aware that patients, and the public, place considerable value on treatments which offer the possibility of extending life when we are close to death. We believe that we should reflect that view when we are asked to make recommendations on the use of medicines that are designed to extend life, at the end of life.”39

However some stakeholders did not share these views, stating that the End of Life Medicines Guidance of 2008 gives unequal weight to those with terminal disease, and if we place a lot of weight on end of life, we may end up with a healthcare system diverting its resources to end of life care, rather than treating curable illnesses and preventing them.

This is a delicate matter. Of course it makes sense to invest more in more common and curable illnesses and in prevention, but this begs the question of whether the minority group comprised of patients suffering from rare or lethal illnesses is not just as deserving as those suffering from hypertension or diabetes. These patients have once held employment and paid taxes, thus contributed to the healthcare of others. It seems only fair and equitable that when it’s their turn, no matter how particular or costly their needs are, they should be looked after. Furthermore, as our society ages and cancer rates increase,55 this minority may become a majority.

Summary of interviews and research

The complex nature of healthcare and uncertainties play a major role in the difficulties encountered in pricing new medical technologies, but asymmetry of information is especially powerful, exacerbated by the involvement of four parties: the manufacturer, the payer, the agent (prescriber) and the consumer (patient). Along with soaring healthcare expenditures, these factors pose a need for, and challenge of regulation of pricing and reimbursement for new pharmaceuticals and medical devices.

In a universal healthcare system such as the NHS, pricing and expenditure are regulated via various reimbursement mechanisms. These have been evolving alongside the healthcare environment, to ensure positive health outcomes alongside production of and access to innovative new products, despite the fixed NHS budget.

Global companies and global markets, international effects on domestic market dynamics, efficiency, equity and value considerations, and incremental innovation are just a few of the considerations when pricing a new product.

Following its market study on the PPRS, the OFT,2 proposed a complete dismissal of the profit and price controls of the old PPRS, recommending VBP as a new pricing framework. Although it is quite difficult to observe it in other countries in its pure form as recommended by the OFT, VBP seems to be viewed by many as the way forward. In theory, this mechanism combines assessment of value of the new product, incentives for innovation, with cost containment.

Many criticisms of VBP have been put forth, as presented in the previous section.

One of the weaknesses of the pre-2009 PPRS, as outlined in the OFT PPRS report, was that the set price cuts did not discriminate between the different levels of value added by the various products. This statement in itself shows that VBP was not intended as a mechanism to lower prices nor to decrease expenditure on medicines6.

VBP was proposed as a mechanism to bring value for money in healthcare, better incentives for companies to invest, especially in areas with unmet need. Furthermore, the OFT’s proposal was intended in the long run to improve stability and sustainability for the government and industry.6

The OFT’s recommendations were not welcomed by all parties. Some manufacturers were in disagreement with OFT’s proposals, regarding VBP as possibly leaning towards a form of therapeutic reference pricing (where a price has been determined for a group of similar treatments and new medicines/treatments are priced at a similar level unless they can show a greater benefit) with added risk and uncertainty, stating concerns about NICE’s HTA methodology and what exactly is meant by “value”.

Patient groups, on the other hand, regarded VBP as possibly providing quicker access to medicines. These stakeholders suggested fairness and equity to be paramount in any healthcare system, thus proposed greater involvement with patients and patient groups when appraising a new medical technology, in order to provide the widest possible access to the best possible treatment.

Moreover, many stakeholders pointed out that although they may lack medical and economics knowledge, patients know how they feel and hence treatment decisions should be made between the patient and the physician. The patient can express their needs and wants to their doctor, who despite the possibility of supplier induced demand made possible by a third party payer, usually work in their patients’ best interest. As suggested by many, prescribing decisions should be made at a local level, however some stakeholders pointed out that PCTs are not really accountable to local people.

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Parallel importing and top-up payments were issues brought up by medical marketing professionals and the NHS Confederation. The NHS Confederation saw top-up systems as potential challenge, because if a medical technology is available only privately, the result may be a parallel market with expensive drugs that may not necessarily be reflective of value. This could lead to pressure on behalf of patients, for the NHS to cover this technology, despite its low added value. A medical marketing stakeholder suggested that industry, represented by the (ABPI), establish an insurance company for drug treatments outside of normal NHS reimbursement, with the elderly, children and the unemployed having their premiums covered by the DH.

Medical marketing professionals felt that a VBP scheme could give manufacturers the opportunity to prove the value of a new product, but in the end, patients and their needs should be shaping the system. Some suggested that when NICE recommends a new technology, the older one it would replace should be decommissioned, to ensure the added value reaches all users, while saving on any possible costs. We must recognise that NICE also produces Clinical Guidelines, which do decommission old treatment when an improved replacement is available, but these guidelines are not mandatory and are sometimes disregarded by PCTs. A question to be considered would be whether to mandate NICE’s Clinical Guidelines.

Health economists and academics were in agreement that value judgements should centre on the patients’ needs and wants. An economist further suggested that VBP would drive companies to produce truly innovative products and incentivise them to demonstrate value even in smaller patient groups.

The DH and the NHS Confederation focused on the high costs of HTA reviews and on defining and calculating value, but did not reject VBP as a viable option for the UK. In September 2008, shadow health secretary Andrew Lansley expressed his support for a VBP scheme, stating that “the NHS and industry should seek to arrive at pricing decisions which reflect the therapeutic value of a drug and (its) innovative value, while ensuring access for NHS clinicians and patients to treatments which are clinically effective.”

Government agreed that a new system should be developed, focusing on delivering value to the NHS, patients and to the taxpayers.

In conclusion, the OFT challenged the Government to ensure that the PPRS delivers value for money to the NHS, patients and to the taxpayers, that it assists in the uptake of new medicines, that it gives incentive and rewards innovation, while providing stability, sustainability and predictability for industry. The 2009 PPRS addresses these points via flexible pricing arrangements, patient access schemes, price reductions and implementing demand side measures such as encouraging clinically appropriate generic prescribing. Furthermore, the NHS Constitution now offers patients two rights in assisting the uptake of new medicines via local NHS and PCTs. To encourage and reward innovation, an uptake and improvement package was introduced in the new PPRS. In assessing NICE’s economic perspective however, the Government commissioned an independent study by the University of York, to become available later this year.

The onus of evidence is on the manufacturing company. There are challenges and high costs associated with collecting and providing ongoing evidence of the effectiveness of the new product, its long term effects and side effects, its economic and social effects, along with evidence of potential new indications of use. There are however, also many benefits and opportunities for manufacturers in a VBP scheme. In theory, the new 2009 PPRS offers possibilities for price increases, and thus rewards for truly innovative products.

It seems that CEA and the QALY are the issues not yet fully resolved. Last autumn, the ABPI stated that they agreed with Andrew Lansley’s view of delivering value for money, but expressed a demand for NICE to be more flexible in its assessment of new medicines.
Chapter 6

6. Conclusion

There was strong agreement from stakeholder groups when ‘valuing medical treatments’ that ‘value’ must mean the costs and benefits to the patient, their families and carers, wider society, employers and the economy. There is a serious disconnect between NICE’s remit and what stakeholders ‘value’.

There is concern from our consultation and our interviews with stakeholders that the NICE and the methodologies used do not represent the different needs of different patients.

“The has been no proper consultation or debate around QALYs”

The NHS is essentially for people and this is very well understood by clinicians, but economic models are very impersonal and do not accommodate the individual needs from different patients. As an example it is important to most working people that they return to work as soon as possible after treatment, particularly as most people are actually employed by small companies and many people work for themselves. NICE does not consider this. The models used to assess QALYs have been criticised for including incontinence and other important health issues.

The economic definition does not seem to take into account the fact that health expenditure has increased considerably recently and in fact in the last two years the NHS has had a surplus of £1.7 billion each year. The rather ‘remote’ economic model - when a new technology is approved by NICE that one patient’s gain is another’s loss, does not appear to take addition funding and NHS surpluses into account.

There is no accepted definition of value-based pricing so 2020Health.org’s definition based on our consultations is -

The price that reflects the value to patients, carers, society and the economy which delivers health benefits that exceed the health predicted to be displaced both elsewhere in the NHS and in the welfare economy, due to their additional cost.

The price of a pharmaceutical or medical technology treatment should properly reflect its value.

Using our definition value-based pricing is to be welcomed and should be the basis for pricing for the NHS.

6.1. The Implications of Introducing VBP Concept and NICE Value Appraisal

The purpose of the PPRS is to allow regulation of prices and reimbursement of pharmaceutical products in the UK, with the main goal of the Government obtaining value for money and giving incentives for innovation.

It has been shown in Figure 3 and Appendix 2 that UK pharmaceutical prices are among the lowest in Western Europe and USA, so it has achieved its purpose. However, if prices in the UK fall too low it will discourage companies from investing in the UK.

Whether this new PPRS scheme is successful depends ultimately on the value and then price attributed to a QALY and its ‘threshold’. If this is raised to encourage innovation then companies would have an incentive.

It could encourage companies to develop more innovative products for the benefit of all. There is a cap on the possible price rise of 30%, which is questionable.

If this is an attempt at cost cutting then the consequences are likely to be:

- Higher prices at launch to cover higher costs from
  - Additional NICE appraisals
  - Further delays to adoption and use of the innovative treatment
  - Greater pricing instability, as list prices may be reduced post-launch thus affecting ROI
  - Considerably more expense and bureaucracy for medical companies
  - An impact on world prices as the UK is used for international price benchmarking thus -
  - Some companies may choose to launch late in the UK – leading to
    - A reduction in UK clinical trials and research
    - Commercially funded clinical research in UK – may be reduced
    - New treatments would not be available in the UK or be delayed
    - Reduced employment in UK from medical companies
  - Parallel Exporting and supply problems for UK patients
  - Further delays in patient access to new medical treatments
  - A reduction in health outcomes for UK citizens

Higher Prices

Value-based pricing per se would be welcomed. Innovative new treatments offering considerable health benefits to patients, their families and carers, society, their employers and the economy should be highly rewarded.

However the system of a series of NICE assessments of cost-effectiveness and then ‘value’ will introduce considerable instability and increased cost significantly. When companies plan launches and pricing they forecast their likely returns to fund their investment. This would introduce greater uncertainty. Initial prices would be likely to increase significantly to counter the increased costs, instability and delays in treatment adoption that would almost certainly follow.

Higher Costs

The costs of a NICE assessment are greatly underestimated by Government, DH and NICE. A medical company bringing a new treatment to market must satisfy MHRA that it is safe and effective by producing evidence from a number of clinical trials. The requirements have been harmonised world-wide and this process continues.

Economic studies for NICE are additional to the licensing requirements and are very expensive. It is estimated that the economic studies required for NICE cost approximately an additional £4.5 million. On top of this there is the cost of the NICE process itself of approximately £350,000 for each assessment (ABPI). Establishing ‘value’ to stakeholder groups is likely to cost even more.

In addition, HTA assessments and NICE technology appraisals can take years and often do. If there is an appeal and court case the process is even longer. If a new treatment only has 8 years patent life left at launch, there is little time to carry out more than one assessment.
Chapter Seven

7. Recommendations

We pose some important questions and recommendations:

7.1. Questions

Our Goal for Health Outcomes?
The overriding goal for the NHS is to improve health outcomes for UK citizens, for society and for the economy. Health outcomes should at least be on a par with other leading world economies. We recommend going further that the UK should be at the leading edge. This would accrue benefits not only to patients, their families, carers and the economy, but also people working in the NHS and research and companies.

There should be a national debate about our goals for health outcomes and overall welfare.

- Should they be set at the leading edge of major world economies or should we aim for health outcomes on a par with our economic comparator countries or is it acceptable to UK citizens that our health outcomes are well below our economic comparator countries as the NHS is funded by taxation?

- Should NICE’s remit be extended to help to ensure health outcomes at the leading edge of world health outcomes with an even greater emphasis on clinical excellence?

- Should NICE take into account any disparity in our health outcomes and availability of the treatments in other leading EU countries when making an assessment and recommendations?

- How can value be driven through the system to improve uptake of new treatments and improve patient outcomes?

NICE produces many excellent reviews improving care pathways. PCTs have little obligations to implement these.

- Should NICE’s clinical guideline implementations be made a mandatory minimum standard for PCTs as their Technology Appraisals are mandatory at present?

- Should there be much more local scrutiny of PCTs to ensure implementation of NICE recommendations as a minimum and to improve health outcomes?

- In order to try to accommodate the differing needs of different patients should clinicians be given more flexibility to make decisions about appropriate treatments with their patients?

- Would practise based commissioning facilitate this within overall budgets?

R&D in the UK

At present the UK is one of the first group of countries where new pharmaceuticals are launched. This also means that many clinical trials are carried out in the UK and the industry also funds fundamental research at UK universities and the NHS. Many people are also employed by pharmaceutical and medical device companies in the UK.

If the UK were to become so unattractive that UK launches were later then it is very likely that these trials and the research would be carried out in another country. The cost of R&D has recently caused many companies to merge and to reduce staff.

Parallel Exporting

Low UK prices also encourages parallel exporting – exporting drugs out of the UK by pharmacies and dispensing doctors, causing shortages in the UK, as has been experienced recently.41

World-Wide Price Benchmarking

As the UK is used as a price benchmark country by other countries a low price would affect world-prices. As one of our respondents said in our survey, if a company can command a high price in the USA and a very low price in the UK, why would they want to sell to the NHS? It would potentially lower their price in the USA.

There must be partnership between medical companies and the NHS. One cannot operate without the other. There must be a fair exchange taking into account true value.
7.2. Recommendations

A public debate on valuing medical treatments

In the UK, healthcare and hence the NHS is funded by taxpayers. This includes individuals and companies. Given the new PPRS' goal of delivering value for money, there is a need for public debate on what value is, and what UK citizens find truly valuable when it comes to new medical technologies. There is clearly a large disparity between what the NHS 'values' and what patients, their families, their employers, the economy and society value.

We have started this debate with our consultation on how we value medical treatments and it does not stop at value to the NHS.

We acknowledge that NICE has created the Social Values Judgements document and the Citizens Council, to better enable the appraisals committees to take account of social values and ethical issues. Furthermore, 2008 brought us the End of Life Medicines Guidance; while this summer the Government introduced the Innovation Pass. There is progress taking place, but we identified that there is still need for greater transparency within NICE’s decision making process.

NICE Technology Appraisal Processes

We recommend the following changes:

- Wider Social and Economic Costs and Benefits Should be included
- The QALY Should be Reviewed
- Categorising Diseases - 5 different categories of disease to be regarded differently:
  - Acute diseases e.g. infections
  - Chronic – long-term disease
  - End of life disease
  - Rare disease
  - Paediatric disease
- NICE thresholds should be reviewed
- Medical technologies and diagnostics should have redesigned appraisal processes
- NICE appraisals should be subsidised, especially for smaller companies
- Price cap increase review
- Explanation of different prices for different indications

Increasing the patent life of medicines should be explored with EU.

Wider social and economic costs and benefits should be included

Contrary to Professor Sir Ian Kennedy’s recommendation, we urge the Government to support NICE in finding means to include wider social and economic considerations, when assessing the value of a new medical technology, as we found in our consultation on ‘valuing medical treatments’. These considerations include:

- Patients can hold employment, hence paying taxes
- Carers can hold employment, hence paying taxes
- Patients need and use less social and informal care
- Patients already holding employment have a reduction in absenteeism
- Companies and the economy as a whole need an NHS which takes into account productivity and helps to reduce sickness absence

We understand that such calculations are very difficult and surrounded by some uncertainty. We also understand that currently, inclusion of such benefits is outside NICE’s remit, but we are encouraged that the NHS has commissioned research into how appraisals may take account of social values and their costs and benefits.

Patient & social costs need to be included and the assessments changed so these are transparent. Value to patients, carers, employers and companies must be considered:

- Effectiveness & efficacy of the treatment (i.e. does it work)
- Safety of treatment
- Health outcomes of treatment - successfulness of treatment
- Side effect profile - few or many negative side effects
- Complications, readmissions and costs of these
- Patient time to feeling completely well/return to work
- Loss of dignity and individualism
- Cost to patient - side effects of treatment e.g. nausea, travel etc
- Invasiveness of treatment e.g. alternatives to major surgery
- Ability to treat more severe or new diseases, especially rare ones
- Care needed from friends and family and social care
- Primary Care vs. outpatient vs. inpatient - where treated - home, GP, hospital & length of hospital stay etc.
- NHS price and treatment cost
- Convenience of treatment for patient i.e. tablet once a day/at home
- Cost to the economy - non-productivity/sickness benefit etc.
- Costs to employer of sickness absence etc.
- Research, development & manufacturing costs
- Cost of clinical trials & economic studies for NICE
- Cost of registration/CE marking & post launch surveillance
- Marketing, sales, launch & distribution costs
The QALY should be reviewed
We recommend that the process of elicitation of a QALY be reviewed. The parameters measured by the EQ-5D questionnaire are especially in need of revision. (Why is mobility so highly rated and incontinence is not formally included in this assessment? One stakeholder expressed his opinion that there should also be a negative value for the QALY, for there are health states that many patients would consider to be worse than death.) We do not advocate the dismissal of the QALY, but rather we suggest that relaxing the decision making process, but strengthening the evidence. As one stakeholder put it, “a QALY is only as good as the data that goes into its making”.

We recommend that when a new product is appraised in comparison to the gold standard already on the market, its incremental value be taken into account; otherwise there is a fear that a standard Therapeutic Reference Pricing system will be implemented in the UK. (For example, a new drug with the added benefit of convenience to the patient, in terms of its administration schedule, should be credited for its incremental innovation, despite its therapeutic similarity to others in its class, and despite the lack in change in treatment outcome. Of course, one could argue that such incremental innovation as convenience to the patient is considered by many as insignificant, but it could also improve patient adherence to treatment, and lowering overall healthcare expenditures for that patient.)

We believe that all a QALY is not a QALY is not a QALY. We recommend that the age, gender, medical history and health status at the time of elicitation, should all be factors taken into account when constructing a QALY.

Categorising Diseases
Different diseases need to be looked at differently, as with ‘end of life treatments’. We propose 5 different categories of disease to be regarded differently:

- Acute diseases e.g. infections
- Chronic – long-term diseases
- End of life diseases
- Rare diseases
- Paediatric diseases

NICE thresholds should be reviewed
We support the House of Commons’ recommendation of a review of NICE’s threshold levels. There is much speculation regarding its origins, its basis when compared with the NHS tariff – the costs for other NHS medical and surgical procedures and its use in an HTA.

We recommend that further research into NICE’s threshold be undertaken to determine whether different illnesses and different patient attributes call for flexibility in the threshold. These have also not increased in real terms.

- The cost per QALY estimates for a technology produces a range of estimates for any one technology. How could this be used to determine a single price?

Medical technologies and diagnostics should have redesigned appraisal processes
Specifically regarding medical devices and diagnostic technology we recommend the DH commission research on how to properly evaluate and appraise such products – the QALY is not very appropriate in these cases, for there is no definite expected outcome. As stated in Kennedy’s recommendations medical technologies differ from pharmaceuticals and should be assessed differently e.g. randomised controlled trials are not always appropriate.

NICE appraisals should be subsidised
We recommend that the Government financially subsidise companies, especially small companies, with the costs of NICE appraisals, in a risk-sharing manner – if a small company believes their new product to be novel and innovative, DH can help finance NICE’s requirements for an appraisal.

Price cap increase review
Under Flexible Pricing, the 2009 PPRS poses a cap of 30% increase over the list price, for new medicines for which there is positive evidence of increased benefit over time.

- We question whether this would give enough incentive.

Explanation of different prices for different indications
If a new indication shows greater ‘value’ the price can be increased for the new indication, but not existing ones.

- We recommend that DH responsible for PPRS explain how this could work?

Increasing the patent life
The patent life of a pharmaceutical is normally only 8-10 when a new drug is launched. This gives little incentive to do further development work because the company would be unlikely to see the benefit. Clinical trials tend to take 1-2 years and there are further delays in publishing etc.

Copyright on books has recently been extended to 50-100 years after the author’s death. Increasing the patent life could lead to greater stability for the company and an increased time to recoup investment in research. In turn this could enable price reductions to be considered. It could also stimulate more development in different disease areas and patient groups and encourage development of innovative treatments for rare diseases.
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Appendices

Appendix One
Analysis of Results from the 2020Health.org Consultation - ‘How do we value medical treatments?’

Appendix Two
Pharmaceutical Prices Comparisons Across Leading Economies – by kind permission of ABPI.
Appendix One
Results of the 2020Health.org Consultation - ‘How do we value medical treatments?’

We will be asking about ‘value of medical treatments’ from a number of perspectives, so please could you let us know the one description that best defines your background.

<table>
<thead>
<tr>
<th>Answer Options</th>
<th>Response Percent</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient</td>
<td>24.2%</td>
</tr>
<tr>
<td>Carer</td>
<td>1.6%</td>
</tr>
<tr>
<td>Patient Group</td>
<td>5.5%</td>
</tr>
<tr>
<td>Patient advocate</td>
<td>2.2%</td>
</tr>
<tr>
<td>Academic</td>
<td>3.3%</td>
</tr>
<tr>
<td>Other (please define below)</td>
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</tr>
<tr>
<td>NHS hospital doctor</td>
<td>4.4%</td>
</tr>
<tr>
<td>GP</td>
<td>9.9%</td>
</tr>
<tr>
<td>Nurse</td>
<td>7.1%</td>
</tr>
<tr>
<td>Private sector clinician</td>
<td>1.6%</td>
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<tr>
<td>Pharmacist</td>
<td>2.7%</td>
</tr>
<tr>
<td>Technician</td>
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<tr>
<td>NHS manager</td>
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<tr>
<td>Private sector manager</td>
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<tr>
<td>Pharmaceutical company manager</td>
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<td>Medical device company manager</td>
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<tr>
<td>Biotechnology company manager</td>
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<tr>
<td>DH manager</td>
<td>0.0%</td>
</tr>
<tr>
<td>Other (please specify)</td>
<td>11%</td>
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</table>

Others
Professions allied to health          5
Patients, patient groups              2
Management Consultants                5
Pharmaceutical/medical company employee 6
### Valuing medical treatments is more difficult. Thinking as a PATIENT, CARER or FAMILY MEMBER OF A PATIENT what costs and benefits would you consider when ‘valuing’ medical treatment? Please tick the boxes you feel add or detract from the value of a medical treatment and add any others that you feel are important to consider.

#### Benefits
- Effectiveness of the treatment [i.e. does it work] 93.8%
- Safety of treatment 89.2%
- Minimal side effects both short & long term 70.8%
- Making own decisions about treatment 60.0%
- Outpatient instead of inpatient treatment 53.8%
- Feeling supported 52.3%
- Becoming knowledgeable about the condition 52.3%
- Feeling completely well 50.8%
- Expert medical backup 24/7 47.7%
- Minimally invasive - alternatives to major surgery etc. 46.2%
- Personalised medicine to suit patients 46.2%
- e.g. Herceptin only works on some patient types, some drugs work better on some people than others 44.6%
- Shorter hospital stay 44.6%
- Local care near or at home 44.6%
- Building relationship with medical team 38.5%
- Quicker acting 33.8%
- Once tablet per day/convenient administration 32.3%
- Helping other patients 13.8%
- Having no scar 13.8%
- Becoming a patient expert 12.3%

#### Costs
- Long term side effects from treatment - infertility, sleeplessness etc. 67.7%
- Complications, readmissions etc from treatment 61.5%
- Time to feeling completely well, back to normal, being effective 58.5%
- Care needed from friends, family and social services 56.9%
- Pain, nausea etc - inability to operate as normal 52.3%
- Time away from work 50.8%
- Fitting treatment into daily routine 50.8%
- Loss of dignity and individualism 50.8%
- Being patronised & not being in control/helplessness 41.5%
- Pain / discomfort and short term effects of treatment 38.5%
- Feeling abandoned & unsupported 36.9%
- Fear of death 35.4%
- Scars, pain & immediate side effects from treatment 32.3%
- Travel to and from treatment & car parking 30.8%
- Multiple prescription charges 24.6%
- Arranging care for family/pets/house 24.6%
- Home care requirements from family or public services 24.6%
- Prescription and treatment costs to reduce side effects e.g. HRT after Hysterectomy 18.5%
- Other - Cost to the health service 1.5%
### Thinking as a Manager in a Medical Company that Supplies Products or Treatments to the NHS: What Costs and Benefits Would You Consider When 'Valuing' Medical Treatment?

Please tick the boxes you feel add or detract from the value of medical treatments and add any others that you feel are important to consider. (Possible benefits are in the left hand column, possible costs in the right hand column)

#### Benefits

<table>
<thead>
<tr>
<th>Benefit</th>
<th>Response Percent</th>
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<tbody>
<tr>
<td>Effectiveness of the treatment [i.e. does it work]</td>
<td>92.1%</td>
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<tr>
<td>Efficacy of the treatment [beneficial effect]</td>
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<tr>
<td>Safety of treatment</td>
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<tr>
<td>Improving health outcomes</td>
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<tr>
<td>Improving patients’ quality of life</td>
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<tr>
<td>Number of patients who could benefit</td>
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<tr>
<td>Side effect profile</td>
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<tr>
<td>Convenience of treatment for patients</td>
<td>60.5%</td>
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<tr>
<td>Shorter hospital stay</td>
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<tr>
<td>Treating patients more effectively</td>
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<tr>
<td>Improving treatment compliance</td>
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<td>Primary care vs. outpatient vs. inpatient treatment</td>
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<td>Helping new groups of patients</td>
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<td>Being a pioneer in new treatment</td>
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<td>Being an innovator</td>
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<td>Treating new diseases, especially rare ones</td>
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<td>Severity of the disease</td>
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<td>Personalised medicine [genetics &amp; genomics]</td>
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<tr>
<td>to suit patients</td>
<td>26.3%</td>
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<tr>
<td>Diagnostic requirements</td>
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<tr>
<td>Opportunity to add value in service redesign</td>
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<tr>
<td>through expanded treatment options</td>
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#### Costs

<table>
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<tr>
<th>Cost</th>
<th>Response Percent</th>
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</thead>
<tbody>
<tr>
<td>Cost of research</td>
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<tr>
<td>Cost of clinical trials</td>
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<td>Manufacturing costs</td>
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<td>Pressure to fund trials</td>
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<td>Pressure to fund education &amp; travel</td>
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</table>
Thinking as a CLINICIAN delivering a medical treatment what costs and benefits would you consider when ‘valuing’ medical treatment? Please tick the boxes you feel add or detract from the value of medical treatments and add any others that you feel are important to consider. (Possible benefits are in the left hand column... possible costs in the right hand column)

### Benefits

<table>
<thead>
<tr>
<th>Benefit</th>
<th>Response Percent</th>
</tr>
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<tbody>
<tr>
<td>Effectiveness of the treatment [i.e. does it work]</td>
<td>95.8%</td>
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<td>Efficacy of the treatment [i.e. beneficial effect]</td>
<td>83.3%</td>
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<td>Good long term safety profile</td>
<td>72.9%</td>
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<tr>
<td>Improving Patient outcomes</td>
<td>70.8%</td>
</tr>
<tr>
<td>Good research evidence that it is effective</td>
<td>68.8%</td>
</tr>
<tr>
<td>Low side effect profile</td>
<td>66.7%</td>
</tr>
<tr>
<td>More effective management of patient</td>
<td>47.9%</td>
</tr>
<tr>
<td>Supporting patients’ treatment decisions</td>
<td>43.8%</td>
</tr>
<tr>
<td>Shorter hospital stay</td>
<td>43.8%</td>
</tr>
<tr>
<td>Primary care vs. outpatient vs. inpatient treatment</td>
<td>35.4%</td>
</tr>
<tr>
<td>Easy dosing regime</td>
<td>31.3%</td>
</tr>
<tr>
<td>Easier access</td>
<td>25.0%</td>
</tr>
<tr>
<td>Onset of action</td>
<td>25.0%</td>
</tr>
<tr>
<td>Treating new diseases, especially rare ones</td>
<td>20.8%</td>
</tr>
<tr>
<td>Local care near or at home</td>
<td>20.8%</td>
</tr>
</tbody>
</table>

### Costs

<table>
<thead>
<tr>
<th>Cost</th>
<th>Response Percent</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost savings as a result of better results of treatment</td>
<td>54.2%</td>
</tr>
<tr>
<td>Severity of the disease</td>
<td>39.6%</td>
</tr>
<tr>
<td>Costs of diagnostic tests required to monitor drug safety</td>
<td>31.3%</td>
</tr>
<tr>
<td>Informing patients</td>
<td>31.3%</td>
</tr>
<tr>
<td>Saving on NHS tariff</td>
<td>29.2%</td>
</tr>
<tr>
<td>Persuading local formulary committees/managers</td>
<td>27.1%</td>
</tr>
<tr>
<td>Changing treatment protocols</td>
<td>20.8%</td>
</tr>
<tr>
<td>Risk management changes</td>
<td>20.8%</td>
</tr>
<tr>
<td>Increased diagnostic tests</td>
<td>20.8%</td>
</tr>
<tr>
<td>Training staff</td>
<td>18.8%</td>
</tr>
<tr>
<td>Clinical adoption protocols</td>
<td>14.6%</td>
</tr>
<tr>
<td>Changing patient pathways</td>
<td>14.6%</td>
</tr>
<tr>
<td>Service redesign</td>
<td>14.6%</td>
</tr>
<tr>
<td>New equipment requirements</td>
<td>14.6%</td>
</tr>
<tr>
<td>Other (please specify)</td>
<td>2.1%</td>
</tr>
</tbody>
</table>

**Other**

“overall costs, vs. overall benefits. So statins .... 50p/ pack £7/ year, to the NHS. NICE priced at £100/ year+, as used drug tariff, not cost to NHS. Benefits... reduced hospital costs £80-£100/ year, but NICE didn’t account for these, which is why we still don’t have these drugs being used in the correct way. To Quote Warren Buffett, risk comes from not knowing what you are doing”
Thinking as a MANAGER IN THE NHS or DEPARTMENT OF HEALTH what costs and benefits would you consider when ‘valuing’ medical treatment? Please tick the boxes you feel add or detract from the value of a medical treatment and add any others that you feel are important to consider. (Possible benefits are in the left hand column...possible costs in the right hand column)

### Benefits

<table>
<thead>
<tr>
<th>Benefit</th>
<th>Response Percent</th>
</tr>
</thead>
<tbody>
<tr>
<td>Effectiveness of the treatment [i.e. does it work]</td>
<td>83.3%</td>
</tr>
<tr>
<td>Safety of treatment</td>
<td>73.3%</td>
</tr>
<tr>
<td>Health outcomes</td>
<td>73.3%</td>
</tr>
<tr>
<td>Shorter/length of hospital stay</td>
<td>50.0%</td>
</tr>
<tr>
<td>Outpatient vs. inpatient treatment</td>
<td>40.0%</td>
</tr>
<tr>
<td>Severity of the disease</td>
<td>36.7%</td>
</tr>
<tr>
<td>No/less long-term side effects</td>
<td>30.0%</td>
</tr>
<tr>
<td>Easy/convenient dosage/treatment regime</td>
<td>30.0%</td>
</tr>
<tr>
<td>Local care near or at home</td>
<td>26.7%</td>
</tr>
<tr>
<td>Speed of action</td>
<td>23.3%</td>
</tr>
<tr>
<td>Invasive nature of treatment</td>
<td>16.7%</td>
</tr>
<tr>
<td>Expert medical backup 24/7</td>
<td>13.3%</td>
</tr>
<tr>
<td>Treating new diseases, especially rare ones</td>
<td>13.3%</td>
</tr>
</tbody>
</table>

### Costs

<table>
<thead>
<tr>
<th>Cost</th>
<th>Response Percent</th>
</tr>
</thead>
<tbody>
<tr>
<td>Price to NHS</td>
<td>73.3%</td>
</tr>
<tr>
<td>NHS staff resource requirements</td>
<td>56.7%</td>
</tr>
<tr>
<td>Longer-term costs, social care etc</td>
<td>50.0%</td>
</tr>
<tr>
<td>Patient pathway costs</td>
<td>46.7%</td>
</tr>
<tr>
<td>NHS tariff</td>
<td>43.3%</td>
</tr>
<tr>
<td>Complications, readmissions etc.</td>
<td>36.7%</td>
</tr>
<tr>
<td>Staff training</td>
<td>36.7%</td>
</tr>
<tr>
<td>Diagnostic &amp; equipment costs</td>
<td>33.3%</td>
</tr>
<tr>
<td>Patient information</td>
<td>33.3%</td>
</tr>
<tr>
<td>Waiting times</td>
<td>20.0%</td>
</tr>
<tr>
<td>Patient throughput &amp; case mix</td>
<td>16.7%</td>
</tr>
<tr>
<td>Formularies &amp; new product appraisal</td>
<td>13.3%</td>
</tr>
<tr>
<td>Home care visits</td>
<td>10.0%</td>
</tr>
<tr>
<td>Other - a new opportunity to defray co-lateral costs</td>
<td>3.3%</td>
</tr>
</tbody>
</table>
Now thinking as an individual citizen, how would you measure the value of a medical new treatment? Please rate the parameters above from 1 to 6 – 1 being most important, 6 being least important.

<table>
<thead>
<tr>
<th>Answer Options</th>
<th>1 Extremely important - must be considered</th>
<th>2 Very important</th>
<th>3 Quite important</th>
<th>4 Neither important nor unimportant</th>
<th>5 Not at all important</th>
<th>6 Should not be considered</th>
</tr>
</thead>
<tbody>
<tr>
<td>Effectiveness &amp; efficacy of the treatment [i.e. does it work]</td>
<td>114 (89%)</td>
<td>12 (9%)</td>
<td>1 (1%)</td>
<td>0 (0%)</td>
<td>0 (0%)</td>
<td>0 (0%)</td>
</tr>
<tr>
<td>Safety of treatment</td>
<td>87 (68%)</td>
<td>33 (26%)</td>
<td>3 (2%)</td>
<td>1 (1%)</td>
<td>0 (0%)</td>
<td>0 (0%)</td>
</tr>
<tr>
<td>Invasiveness of treatment e.g. alternatives to major surgery</td>
<td>18 (14%)</td>
<td>41 (32%)</td>
<td>55 (43%)</td>
<td>7 (5%)</td>
<td>0 (0%)</td>
<td>0 (0%)</td>
</tr>
<tr>
<td>Side effect profile - few or many negative side effects</td>
<td>27 (21%)</td>
<td>64 (50%)</td>
<td>29 (23%)</td>
<td>2 (2%)</td>
<td>0 (0%)</td>
<td>0 (0%)</td>
</tr>
<tr>
<td>Health outcomes of treatment - successfullness of treatment</td>
<td>80 (63%)</td>
<td>35 (27%)</td>
<td>7 (5%)</td>
<td>0 (0%)</td>
<td>0 (0%)</td>
<td>0 (0%)</td>
</tr>
<tr>
<td>Convenience of treatment for patient i.e. tablet once a day/at home</td>
<td>12 (9%)</td>
<td>29 (23%)</td>
<td>65 (51%)</td>
<td>14 (11%)</td>
<td>2 (2%)</td>
<td>0 (0%)</td>
</tr>
</tbody>
</table>
Appendix One

Now thinking as an individual citizen, how would you measure the value of a medical new treatment? Please rate the parameters above from 1 to 6 – 1 being most important, 6 being least important.

<table>
<thead>
<tr>
<th>Answer Options</th>
<th>1 Extremely important - must be considered</th>
<th>2 Very important</th>
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<th>5 Not at all important</th>
<th>6 Should not be considered</th>
</tr>
</thead>
<tbody>
<tr>
<td>Loss of dignity and individualism</td>
<td>32</td>
<td>41</td>
<td>30</td>
<td>11</td>
<td>5</td>
<td>1</td>
</tr>
<tr>
<td>Cost of clinical trials &amp; economic studies for NICE</td>
<td>12</td>
<td>22</td>
<td>38</td>
<td>18</td>
<td>18</td>
<td>12</td>
</tr>
<tr>
<td>Cost of registration/CE marking &amp; post launch surveillance</td>
<td>10</td>
<td>17</td>
<td>35</td>
<td>32</td>
<td>14</td>
<td>12</td>
</tr>
<tr>
<td>Marketing, sales, launch &amp; distribution costs</td>
<td>7</td>
<td>10</td>
<td>33</td>
<td>31</td>
<td>25</td>
<td>19</td>
</tr>
<tr>
<td>NHS price and treatment cost</td>
<td>23</td>
<td>32</td>
<td>40</td>
<td>16</td>
<td>7</td>
<td>4</td>
</tr>
</tbody>
</table>

Other (please specify)

1. Cost to patient on prescription charges for possible multiple items over an extended period and the inconvenience of obtaining this medication under current PCT guidelines.
2. No post-code restrictions.
3. Quality of life.
4. Need to consider all costs.

Appendix One

Now thinking as an individual citizen, how would you measure the value of a medical new treatment?

Please rate the parameters above from 1 to 6 – 1 being most important, 6 being least important.

![Bar chart showing ratings for different parameters](chart.png)
Appendix One

Are there any specific characteristics of different types of disease that would affect your perception of the "value" of a product designed to treat this condition?

FOR A SHORT-TERM DISEASE SUCH AS FLU e.g. you may be prepared to put up with more side effects from the treatment as the disease doesn’t last long - PLEASE COMMENT

- N/A
- Short term non-life threatening, would be prepared to put up with infection.
- So long as life threatening symptoms are removed.
- Acute non-serious disease, may just tough it out.
- Consequences of not treating the disease
- Rapid recovery from a minimally invasive technique as long as it’s safe and effective is very important.
- Life saving, preserving QoL, preventing severe chronic symptoms
- Provision of care for the patient during this period. Ability of patient to work during this period.
- Independence and privacy of patient during this period.
- Possibly.
- If it made me very sick.
- Self-funding.
- Able to continue working while symptomatic.
- Unpleasant side effects can be tolerated if there is a short time period and an end point.
- Agree - short term discomfort is outweighed by benefit.
- Quick resolution.
- Agree as long as safety was not affected.
- If the disease is going to get better quickly then it might not warrant treatment.
- Agree.
- Consider using drugs apart from Paracetamol for flu useless.
- Value for short term conditions is speed of treatment.
- Would prefer to have as little treatment as possible.
- All side effects should be known.
- Impact on quality of life is key.
- More important is the severity of the disease.
- It would depend on what the side effect was.
- Better to do without for self-limiting illness.
- Efficacy and speed of symptomatic relieve.
- Not at all, I’d rather go without medication.
- For a short term disease I may wish to have simple symptomatic treatments. I certainly would not want.
- Something with many and/or potentially serious side effects if I am going to recover quickly anyway.
- Great effectiveness at cheapest price (side effect profile and monetary).
- I would stick to treatments with which I am familiar.
- Would not put up with side effects of treatment.
- Everyone should be able to tolerate effects of flu unless other illness will be affected.
- People tend to take drugs as soon as they have a slight sore-throat, they forget that every drug can have side effects.
- Within reason side effects are common with most preparations, it’s how it affects you that counts, as side effects usually indicates the drug is working.
- I am happy to self treat for such things as flu by purchasing paracetamol based medicine from chemist, supermarket.

- Yes
- No. With self limiting illness I would most likely discontinue if side effects were significant.
- Want to clear up as quickly as possible, yes could live with short term side effects as long as not debilitating.
- Expect the product to act quickly.
- Fast acting.
- Given the current pandemic, I believe I would be prepared to take a short term course of treatment to ensure that my overall wellness was maintained.
- No sense in medicating for low level short-term illness, especially if treatment is prone to side effects.
- Acute life-threatening conditions should be treated aggressively.
- Not bothered about convenience or side effects if short term although effectiveness important.
- Serious side effects versus disease symptoms.
- Willingness to tolerate side-effects would depend on severity of disease - if it’s not very severe, wouldn’t want to have side-effects.
- Yes, this is true.
- I may be more tolerant of side effects but to be honest I don’t want any short or long term.
- Not really as I’d rather ride the flu out than have a side effect that made me feel worse.
- For diseases of this sort I probably would not take any medicine.
- Would want absolute recovery - prepared to put up with short term pain or other inconvenience.
- Not sure treatment is needed for minor disease unless the patient is in a selective area of concern.
- Yes, quality of life affected would be short lived for a cure.
- Agree - we are more likely to put up with side effects for a shorter period.
- Value is determined by speed of access to treatment.
- Would choose drug with least side effects.
- Duration of action and side effects worth it short term for longer term gain.
- No unnecessary side effects should be acceptable.
- Not keen on any side effects but if I was sure it would do the job I could tolerate it for a short time.
- Would not expect side effects from medication for 'trivial' health issues.
- Provided side effects not as debilitating as illness.
- Yes if less than a week.
- Yes.
- Would not tolerate side effects unless the treatment vastly shortened the term of the illness; for example, I would rather have Flu for two weeks rather than for one week but with many potential side effects.
- Value on: fast acting, able to carry out daily activities.
- Cost and safety profile.
- Depends on severity of disease; palliative treatment may be sufficient.
- Yes if it is a serious short term illness.
- Does it save peoples’ lives?
- Would need to improve symptoms and length of illness.
- A stoical attitude should be encouraged!
- No.
- Yes.
- Must be cheap.
- Flu is a self limiting disease so does not need ‘treating’ as such.
- I agree.
- But if it is self limiting why take anything at all?
- Yes.
- Depends upon the severity.
What benefit from treatment if short term disease
More side effects would affect compliance.
Side effects long term.
Part of normal life.
Most people do not analyse cost/benefit/risk/side effect in long term short term they want instant quick fix.
No, I would not be prepared to have side effects.
YES.
Would only accept minimal side effects for a self limiting disease.
AGREE.
Encouragement of self medication rather than resource based treatments.
Would not want to feel worse during treatment.

Are there any specific characteristics of different types of disease that would affect your perception of the "value" of a product designed to treat this condition?

FOR A LONG TERM CONDITION SUCH AS DIABETES e.g. having to monitor yourself each day and take treatment each day may make you more concerned about side effects, ease of use and convenience of the treatment - PLEASE COMMENT

N/A
Treatment, effectiveness, ease of use, ease of access and costs important.
This should be tailored for the patient and where possible the person should have independent care in their homes.
Inconvenience would be important, but patient more so.
The side effects of hormone treatments for some pain conditions are significant.
Convenience of testing and taking the treatment.
Importance of sustaining dignity, self-sufficiency/independence, sensory faculties.
Want effective medication that enables me to continue working for a living.
Important to raise own responsibility
Simple to use so that I could “forget” I had a long term condition.
It makes you more ‘paranoid’ and therefore hinders treatment which is otherwise about subtle changes.
Completely agree - making life as easy as possible including treatment regime, aids to enhance compliance.
Ease of use become paramount importance. Anything to support patient leading healthy lifestyle.
Better quality of life.
I would put up with some inconvenience to improve quality of life.
Less interference with lifestyle.
Important in patient compliance.
Diabetic drugs improve and extend quality of life for patients, high value for improving life apart from discomfort and continuous monitoring.
For long term conditions value represents the ability to live as normal and care free life as possible - ease of use, lack of side effects and interactions and ability to work / be independent.
Slowing or delaying long term outcome decline.
Ease of use of treatment and monitoring of disease.
All side effects should be known.
The more self treatment and control I could have the better.
TRUE
I would put up with anything and do anything to avoid having to give myself injections. I would rather be com.
Are there any specific characteristics of different types of disease that would affect your perception of the "value" of a product designed to treat this condition?

FOR A TERMINAL DISEASE SUCH AS CANCER e.g. as the disease is more serious you may be prepared to put up with more side effects from treatment - PLEASE COMMENT

- N/A
- If product saves lives or seriously improves quality of life when has been very poor, worthwhile, but only to a limit - probably quite individual.
- Everything that is possible should be done for people with cancer with no regard for cost.
- Side effects of treatment less important.
- It is for the patient to decide whether the benefits outweigh the risks based on as accurate information as possible including data from negative trials.
- Quality of life and extra time to make final arrangements.
- Quality of Life is very important and palliative treatments should be as minimally invasive as possible to achieve the best palliation.
- Sustaining control, independence, mobility and cognitive ability.
- Patients’ dignity in dying. Support and guidance available.
- More informed choice of options.
- Possible to self administer, at home, stay in control of treatment.
- Often someone will tolerate chemo which can be foul because of a hope that it may work.
- As long as there is some assurance that treatment will cause remission or cure. I believe patients are not.
- Fully informed or engaged about this and may believe that there is a guaranteed cure. Patients must be given the choice as to whether they go through treatments which are aggressive.
- Better quality of life.
- I would want the end to justify the means.
- Agree.
- Most patients expect a certain level of discomfort with cancer and bear it until it becomes unmanageable level. Drugs must be essential for pain and reducing symptoms and must be worth it. It seems sensible that very expensive medication that only extends life by months needs careful allocation to PT groups.
- For terminal diseases value is pain free / life extension.
- Put up with some side effects if treatment likely to be successful.
- All side effects should be known.
- Again the more control a treatment gave me the more I would value it.
- True but only up to a point where quality of life is still reasonable. Not life at all costs.
- At this moment in time I think I would rather be kept comfortable than put up with uncomfortable treatment.
- Quite right.
- Effective without loss of independence and later of dignity.
- Depends on my age and family circumstances.
- If I have a terminal condition I would not want any treatment that would be unpleasant unless it was to significantly prolong my quality of good life.
- Efficacy - is it worth it for those extra few days/weeks/months - depends on how effective. Quality of life more important than length.
- Probably.
- One would have to, to a degree.
- Most patients will tolerate side effects if they believe the treatment is being effective.
- Cancer is not necessarily more serious than diabetes. It is just perceived to be. Depends of your age, for some elderly chemotherapy is not a solution.
Yes, if the benefits are worth it.

Quality of life over quantity or at the very least a favourable balance would be of greatest value.

Value placed on: lengthening life, pain relief.

Only if survival is increased in a meaningful way.

Yes, if treatment is effective and can gain years with good quality of life.

Possibly the opposite. If I am dying I attach more importance to comfort and less to duration of life.

What is the cost/benefit?

Would not be as concerned re long term side effects. Looking for effectiveness on symptom management.

Need to balance interventions against quality of life issues and patient choice.

Agree.

Balance - depends on prognosis and side effect profile.

Benefits must be in quality of life not quantity of life.

Efficacy is of paramount importance, increase side effects may be the cost.

Not necessarily, may deter some pt’s from treatment.

Agreed.

If it’s effective and assures good outcome.

No as it may affect quality of life.

Quality of life - would rather have short life and good quality than long life with poor quality.

Expectation of side effects, but must be minimised.

No pain.

A good death is better than a prolonged life.

Think we should have a serious debate and following the Swiss provide more end of life choices.

Some of the side effects are so unpleasant that I would need to be convinced of the benefits to put up with them.

YES.

Would accept severe side effects in an attempt for cure but less for palliation.

AGREE.

Patients tend to accept major side effects in search of cure.

I am not sure that I would want to suffer more with treatment if outcome not known.
How would you ensure delivery of good value medical treatments now and in the future? e.g. do you think individual patients should have an input into this? Do medical companies do this anyway when they price new treatments? Should a Government body oversee this?

Should this be done at a local level? Should it be decided by your doctor? NHS Manager?

PLEASE COMMENT

- N/A
- Patients should have an input. Drug companies need to be there to advise - but NO more. Local area etc. all need an input because of costs and who will prescribe/monitor... I think an independent body needs to assess and collate all views and then respond.
- The price should be set by the government, cheap loans could be given by the government to cover R&D costs to cover companies until the treatment is used sufficiently within the population to cover production costs.
- Hospitals and PCTs should shop around for best value.
- Patients and patient representatives should have a major role in this. NICE is the organisation that is best placed to do this - it is reviewing its processes and seeking to improve how it does this.
- Allow pharma companies to get more involved in healthcare delivery.
- More new treatments should undergo Quality of life and cost effectiveness studies with patient input.
- Ensure availability, awareness and affordability; decisions at individual level; top up for those products either not reimbursed or top up at level over an agreed NICE value level (i.e. not full cost with pharmacy 50% mark-up plus VAT).
- NICE or equivalent.
- Government should stop interfering. Leave it with the experts.
- At a local level by GPs.
- GP should remain in control of these decisions on an individual basis.
- Treatments need to have suitable evidence that they work. Other more 'iffy' things should be taken off prescription and become the territory of the pharmacist and OTC. Tighter rationing is the only way to encourage a partner to reduce secondary care costs.
- Patients at individual level would be very emotive about this. Patient groups or focus groups comprising a balanced group of patients should be used for input. Medical companies are only interested in getting return on their investment - do not believe their costing of a medicine bears any relation to patient needs to the needs of the NHS. This is evident by new medicines being increasingly expensive even though many do guarantee cure.
- Should be done nationally with SOS for health with advice from NICE or other body so no postcode rationing.
- Clinicians as well as doctors and patients should have a significant input into valuing treatments.
- The decision should be led by doctors.
- By a group with medical, paramedical and lay representation.
- Medical companies seem to concentrate on selling the drugs and saturating the market with their users. If drugs were cheaper, more patients would opt to buy their own if they could. A government body to oversee would be good but difficult to manage differing opinions about allocation at local level. Decisions about allocation should not be made by GP, doctor or NHS managers in my opinion. NICE does a good job.
- Co-pay for more low cost treatments to increase funds to high cost new drugs.
- Patients should always be treated as individuals and their wishes and those of their carers should be taken into consideration. There must be informed consent to any treatment. Having a government body which is totally removed from the needs of the individual and which only looks at cost of treatment is not desirable.

Take politics out of medical treatments.

- NICE should be closed and issues about what works should be made at a EU level for macro decisions and at a local level with more effort going into the interaction between Dr and the people they care for making the decision together.
- Patient care must be put back into the hands of local health departments, who are answerable to the public.
- Medical education must retain and build upon ethical issues so that there are successive cohorts of well-rounded, articulate and knowledgeable physicians and surgeons able to make properly balanced judgements.
- Commission of Professionals to determine delivery of treatments passed by UK and other (e.g. FDA) safety authorities.
- A multi-player but transparent body should oversee. If local/individual solutions could have worked as effectively for every group, sum of parts could have been better but it hardly does.
- Compare results with national and international standards.
- I think NICE is a good method for this.
- Dr should decide on clinical evidence. Government pushes up costs.
- Patients need to be educated before they can have an understanding of their treatment.
- If individual patients had a say in costs (other than if they are self paying), it could be very chaotic.
- Why not have a medical financial board do it not as they can better understand costs.
- Some companies do involve patients in development of products. The value of treatments can only be ascertained if research is undertaken to provide evidence. QALYs are not robust enough to incorporate true value in their current form.
- The patient needs the product, so pharmacists shouldn’t be allowed to sell medicines to other countries.
- Decision by patient but after thorough discussion with medic.
- I feel much more should be done on improving the ‘well being’ of the nation and in time this will have an effect on the cost of medicines and treatment.
- Don’t think local level works as there is still discrepancies between England, Scotland and Wales and also at a PCT level e.g. IVF in Oxfordshire is appalling.
- Balance between safety and innovation. Fast-track regulatory approval / NICE will help this.
- Allowing patients to ‘top up’ by paying extra for expensive treatments if they wish. Patients should have involvement into these decisions, but government should be involved also - but at a distance.
- NHS should ideally be apolitical. Decisions should be national basis, but with flexibility to allow for local need. NHS management need to steer decision making but with effective clinical involvement - or clinicians will not be engaged in the outcome.
- Government oversight operating a national policy. Need to avoid post code lottery scenario. Doctors do not necessarily make good managers, there primary interest is patient care therefore value considerations may be reduced.
- Good value should be determined by the purchaser i.e. the taxpayer and therefore patient advocacy groups are vital in analysing ‘good value’.
- Do not involve government at all. A lay body and professional should decide.
- I believe that the prescription of medicines must be to the benefit of all parties. Healthcare development requires funding or there will be no benefit to business in the development of medicines which ultimately are of benefit to patients. However this balance should be moderated.
- Large companies must work with healthcare bodies/governments to ensure that this benefit is managed to a mutually beneficial advantage. I believe that the standard of care throughout the UK should be equal and not subject to local disjoined budgetary constraints, and that the government have a duty to ensure that my treatment is the same standards irrespective of council boundaries or country of residence within the UK. I believe that persons who do not receive benefits (pay for prescription charges) should not face penalties in charges to supplement the unfair systems currently...
in place, and that the process and ease of access to doctors (GPs), consultants, hospitals, in patient and out-patient services should be managed for the convenience of the patient, not bureaucratic processes. I also live in a County - not a State!!!!!!

- Get the NHS to evaluate the many other activities it does - disinvestment from poor value activities that are not cost-effective will release funds for better patient management (more medical support in the home/community setting, better facilities in hospital, etc).
- Delivery of value should be measured by patients, doctors & nurses & government. I don’t see any need for input from medical companies as I think they have their own interests.
- Quicker NICE recommendation.
- Given the low levels of health literacy in this country, I don’t think patient input is appropriate or would actually be very helpful. Views are likely to reflect the educated middle class groups.
- I think medical company + independent medical body.
- I think the public should be prepared to make co payments or insurance. After all some people spend £100s a year smoking and drinking but object to paying for something that may save their lives.
- I think it should be a collaborative effort.
- There should be availability across the NHS and not left to local “post code lottery”. Patient Groups not but individual patients should be involved in determining good value. If it is agreed that the medicines has good value, individual NHS organisations should not be allowed to restrict its use.
- New treatments should only be used by the NHS where they demonstrate an unmet need or are considerably more effective that current medication. ‘Me too’s’ should have very limited markets and should not be more expensive than the generic equivalent.
- Less government interference and more opt in choice
- Much more open dialogue is required between all stakeholders.
- Only a Gov’t body has the bigger picture required to oversee this.
- A Government body should oversee this to ensure equity.
- Patients should be given all alternatives however clinician should explain to patient why they decide to use a particular drug over another.
- Link to health outcomes/improvements.
- Doctor should evaluate the patients needs not an accountant.
- I think getting patients’ views is a very good idea. There needs to be a joint body from the
- Pharmaceutical world together with the Govt to decide on prices. Perhaps more flexibility is required at local levels to suit the needs & demographics of an area. When someone is v ill & is told that they are too expensive they can’t get treatment even though they have paid taxes & contributed all their life, it not right. There are lots of people that ‘use’ the system for their benefit & always receive benefits. I think at present the control has gone too far to the side of the managers & pharmaceutical advisors who don’t see the patients. There needs to be a better balance so the clinicians have more say. I think Drs feel very unmotivated these days as they are told what they can & can’t do. They are in a v good position to know what is best for the patient.
- This should be determined by doctors and consultants who can assess best the potential benefit to each patient.
- Has to be a medical decision on treatment to individuals - no ‘post code lottery’. Governments have healthcare budgets however, so overall costs of medicines needs to be controlled centrally. NHS needs to look at what is treated - plastic surgery/sex changes etc are not essential treatments.
- Government/Nice appears to be driven by cost and political issues. Patients and doctors need to make decisions with all information available.
- No, yes, yes, no, no, yes. There should be national guide lines. Patient group voice with government involvement, not just passive listening. Patient preferences should be central to determining value. Governments have a responsibility to ensure this is the case.

- Individual patients and/or patient groups should be consulted to ensure delivery of good value medical treatments. “Value” extends beyond what is financially of value and must include what is of value to a patient - such as reduced drug side-effects. To ensure this is a fair and transparent process it needs to be overseen by a government body at a national level to ensure consistency throughout England. However, there also needs to be some flexibility to ensure that where a local population has specific needs that these are responded to.
- Stop incremental innovation.
- Patients should have an opportunity to provide input, but many just want to know they are being given the “best” treatment; the problem is understanding what is best for a given patient. Companies do develop health outcomes data, but systems are currently v repetitive and inefficient e.g. repeating data for different regulators.
- There is an implicit tension between local NHS managers making decisions about services and treatments and national NHS policy implying that the decisions should all conform to the same policy. Either we have local decisions (which means local variations) or central decisions (which means local managers can’t be responsible for cost overruns locally). If we have local decisions it would help to have some kind of public input to NHS decision making locally so that the decisions can be made with some local legitimacy.
- I haven’t got a problem with NICE doing this, but then I have just been working on a NICE pathway, but from my previous comments not all that NICE has done has been good. There are problems with the process, and usually the biggest problem is asking the right question.
- Groups representing patients interest.
- Central input is vital as funding must be used appropriately but guidance should involve patients too.
- Patients should be included much more.
- Need patient and public input to a clinician led system.
- Difficult as facts rarely available should be as local as possible. not biased drug information from a reliable researched source.
- Increase patient involvement in local and national decision making. Whole systems approach to decision making.
- Decided by all parties involved more centrally funded research, separate from drug companies.
- Should involve all these.
- Patient in consultation.
- Every person is an individual and decisions should be considered individually by a professional team and patients should be involved.
- Difficult for pts to input as they are directly affected maybe someone who has had the treatment would be better. A government body should oversee to ensure fairness of use of different drugs especially very expensive ones.
- All points of view should be taken into consideration.
- Think NICE should remain to help and support clinical decisions.
- I think this should be done at a national level with input from clinicians and patients.
- Patients currently have little or no control over the price of treatments and this does not appear to be a focused consideration.
- A government body should monitor this in the interest of all not just their client group.
- ETHICAL RESEARCH/ patient input necessary.
- Individual patients have their own agenda so may be a subjective view; as tax payer I want value, care for all, but death comes to us all. Not sure that I agree with funding treatment to prolong life for a few weeks, but then again neither myself nor my family have been in this situation.
A proper review of all new treatments should be held, preferably at national level and taking into account experience in other countries. Patient input should be sought, but the emphasis should be on hard evidence from clinical trials and maybe case study evidence from centres that have a lot of experience in the condition and the new treatment under discussion. Anecdotal evidence is not enough. Pricing is a big issue, drug companies make a lot of money out of others misfortune. If pharma lowered their prices they might find the sums actually add up better.
About 2020health

2020health is a centre-right Think Tank for Health and Technology

Vision

- A healthier nation

Mission

- We want to improve health through effective commissioning, competition and technology.
- We seek a level playing field between the public and private sector as they work to improve health outcomes.
- We search for ways in which the workforce can take more responsibility in local healthcare.
- We examine the consequences of healthcare decisions on society, lifestyle and culture.

Work streams

- Public health
- Technology
- Sustainability