Cancer52 Position Statement on Value Based Pricing (VBP)

Summary

Value Based Pricing (VBP) was proposed by Government in 2010 as a key way to deliver improved access to medicines. But detail is lacking, even though confidential Department of Health (DH) and The Association of the British Pharmaceutical Industry (ABPI) negotiations have recently commenced.

Whilst Cancer52 is always broadly supportive of efforts to improve patient access to treatments and to focus research on innovations that matter most to patients, we have concerns that VBP risks making the situation worse, not better. VBP could lead to delays in the launch of new treatments or even no launch at all; VBP could blunt incentives for clinical research in the UK and VBP could simply leave those with rare and less common cancers behind.

We want to see more research and wider engagement so that Government does not make mistakes with VBP that will affect patients now, and in the future. Cancer52 wants to work with Government on design, implementation and evaluation of the value assessment within VBP so that it can deliver on its promise of improved access, and ensure incentives for innovation on treatments for those with rare and less common cancers.

Who does Cancer52 represent?

52 per cent (recent statistics show an increase to 53%) of UK cancer deaths are from the rare and less common cancers.

Despite this, the rare and less common cancers remain severely under represented and under-funded across all areas, including policy, services and research.

Cancer52 is an alliance of more than 60 charities working to address this inequality and improve outcomes for patients with these highly challenging diseases.[1]

[1] Please see our website for more details, including details of our membership. http://www.cancer52.org.uk

Why is Cancer52 concerned about proposals for VBP?

The Department of Health (DH) set out proposals for VBP in December 2010.[2] The five objectives of VBP are to:

- improve outcomes for patients through **better access to effective medicines**;
- **stimulate innovation** and the development of high value treatments;
- improve the process for assessing new medicines, ensuring transparent, predictable and timely decision-making;
- include a wide assessment, alongside clinical effectiveness, of the range of factors through which medicines deliver benefits for patients and society;
- ensure value for money and best use of NHS resources.

VBP policy and its implications:

The objectives in **bold typeface** above are those that are of key concern to Cancer52. We know from our members' experience and activity that access to treatments can be a problem, and that research efforts to treat rare and less common cancers remains under-funded and poorly coordinated within the NHS.

VBP proposals set out a new approach to assessing the value of new medicines, building on the current cost per Quality Adjusted Life Year (QALY) approach by adding in considerations of:

- Burden of illness (severity and unmet need)
- Innovation
- Wider societal benefits

The presence (or absence) of each of these 'modifiers' could result in a different (potentially lower or higher) cost effectiveness threshold to be applied when considering the value for money of a product. The proposals suggest that the NHS will only pay the price that is in line with this value assessment.

Although not explicitly stated, Cancer52 believes that there is essentially a two stage process in VBP:

1) value assessment and
2) linking price with that value assessment.

[2] http://www.dh.gov.uk/prod_consum_dh/groups/dh_digitalassets/@dh/@en/documents/digitalasset/dh_122793.pdf

Cancer52 believes that value assessment cannot be undertaken without the input of patients and those that represent them.

To proceed without that input presumes a competence attributed to the ABPI to represent the views of patients. The ABPI have yet to signal their willingness to adopt that role.

More details are now long overdue following these initial proposals. Cancer52 has been part of discussions with the DH and although some details are emerging, there remain significant unanswered questions.

For example:

- Is it desirable to include all the value components (such as wider societal benefits)?
- How will the components of value be measured?
- How will each component be aggregated into the value assessment overall?

Cancer52 understands that negotiations between the ABPI and the DH are underway. Cancer52 wants to be part of the design of value assessment, but recognises that the second step, pricing, is a matter for companies and Government.

Particular concerns with VBP policy:

Cancer52 supports all efforts to improve access to medicines and to encourage research that will deliver future medicines. Some elements of VBP could present an opportunity to achieve that ambition: for example by allowing greater weight to be placed on patient focused innovation. However we are concerned that:

· A. **VBP proposals do not address access** for patients sufficiently clearly, leaving considerable uncertainty as to how access will be delivered in practice. This is a key issue when money is short. It is also a concern as the NHS becomes more decentralised as the Health and Social Care Act 2012 reforms are implemented in England. Finally, proposals do not deal with the complexities of increasingly sophisticated targeted therapies and companion diagnostics which are available to treat some cancers.[3] VBP may well lead to lengthy negotiations over the value assessment, and the price commensurate with that value assessment. This has the potential to delay access, or, at worst, lead to no access at all, if it causes commercial pharmaceutical companies to choose not to launch in the UK.

[3] Other terms for "targeted therapies" include personalised, precision or stratified medicines

- B. **VBP proposals could adversely affect future clinical research in the UK and blunt incentives for future innovation**. UK patients may well fall behind their European counterparts if VBP results in longer assessments and lengthy price negotiations with companies. VBP runs the risk of either late launch, or no launch at all. That could result in the standard of care in the UK falling behind that in other countries with the consequence that companies will look to other countries, where there is a definitive standard of care, to conduct their trials. Clinical research is a vital way of developing our combined knowledge, helping identify future targets for research, and in some cases can be one of the few ways that patients can access medicines early. It is vitally important for products to be available to treat rare diseases where building up sufficient sample sizes remains an ongoing challenge.

- C. **VBP proposals are not sufficiently clear on how they will address the specific issues that affect access to medicines for those with rare and less common cancers**. Previous work by NICE has acknowledged, for example, that orphan drugs for the rarest diseases require a different approach.[4] [5] So far the Government has not been clear on whether, and how, VBP will approach such products.

Our concerns are real: there is evidence that these problems exist now and we do not see how the proposals for VBP will counter these. We set out some of that evidence in an appendix.

What does Cancer52 want?

Cancer52 is asking for:

1. More **research should be undertaken to establish what relationship, if any, exists between pharmaceutical pricing and reimbursement and the location of research and clinical trials**. This is to avoid making mistakes now, which will have long term and potentially irreversible impacts on current and future patients.

[4] http://www.nice.org.uk/niceMedia/pdf/smt/120705item4.pdf
[5] EU Regulation on Orphan Medicinal Products http://ec.europa.eu/health/files/eudralex/vol-1/reg_2000_141/reg_2000_141_en.pdf and http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000029.jsp&mid=WC0b01ac05800240ce

2. The **cost effectiveness threshold(s) under VBP should be empirically based** and perhaps set by a new, independent advisory body that sits outside of the existing Health Technology Assessment (HTA) organisations in the UK. This work needs to explicitly test the thresholds that would be needed **to ensure incentives for future research for treatments for uncommon diseases** and ensure that the development of and access to medicines is as efficient as possible.

3. **Full and open approach to further design, implementation and future evaluations of VBP**. This includes full participation of patient organisations in value assessment to help overcome concerns about a lack of involvement to date, not just in VBP development but more generally in decision making.[6] Evaluation should include how far VBP has changed research priorities, including the focus on research to treat those with rare and less common cancers. Cancer52 is willing to work with all stakeholders to build on the opportunities of reform and to improve access to medicines in the UK.

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[6] Barham, L Public and patient involvement at the UK National Institute for Health and Clinical Excellence Patient 2011;4(1):1-10

Appendix:

What is the evidence that supports our concerns?

We provide in this appendix evidence that explores the key issues that concern Cancer52. It is not comprehensive, but we believe it makes it clear that VBP needs to be carefully designed, implemented and evaluated to avoid making current problems worse.

A. **VBP proposals do not address access** sufficiently clearly

Cancer52 knows that patient access to medicines in the UK is the result of a variety of decisions of many different stakeholders: pharmaceutical companies, regulators, and agencies such as National Institute for Health and Clinical Excellence (NICE), Scottish Medical Consortium (SMC) and All Wales Medicines Strategy Group (AWMSG) and local commissioners/budget holders/payers and clinicians. Policies such as the Cancer Drugs Fund (CDF), Patient Access Schemes (PAS) and Individual Funding Requests (IFRs) also affect access. Together they affect what medicines are available, and when they are available, and how consistent access is across the country.

In addition, the Cancer Drugs Fund has an uncertain future.

A.1. There is evidence of **inconsistency in what is available**:

- Newer cancer treatments are increasingly 'personalised' for specific subgroups of cancer patients. It is recognised that these have high R&D costs for small markets. Such treatments are often not recommended for use in the NHS.[7] According to a comparative analysis of accessibility undertaken by Professor Sir Mike Richards CBE, National Clinical Director for Cancer, these treatments may well be available in other countries but less so in the UK, with the UK consequently ranking poorly. [8]

- Worryingly NICE statistics already suggest that some companies are choosing not to put forward their oncology products for appraisal. There have been nine non-submissions from 1st March 2000 to 31st August 2012.[9] This can reflect a host of issues. Whilst access at any cost may not be achievable, it does highlight that the UK is becoming less attractive for development and approval of new therapies**.** And that means fewer chances for UK patients to access medicines that companies may well make available in other countries.

[7] http://www.nice.org.uk/newsroom/nicestatistics/niceandcancerdrugsthefacts.jsp
[8] As cited in www.myeloma.org.uk/about-muk/news/press-releases/myeloma-uk-publishes-opinion-leaders-report-on-value-based-prici/
[9] http://www.nice.org.uk/newsroom/nicestatistics/niceandcancerdrugsthefacts.jsp

- In the first national evaluation of the CDF, the Rarer Cancers Foundation (RCF) found that access to cancer drugs is now considerably greater in England than it is in Scotland, Wales or Northern Ireland. People in Scotland are three times less likely and people in Wales five times less likely to gain access to previously unfunded cancer drugs than their counterparts in England.[10]

- RCF also found that the NHS East of England has included 76 treatments on its CDF priority list, whereas NHS South West has included only eight.[11]

A.2. **There** is **evidence of decisions regarding access to therapies simply taking too long, particularly in cancer**; there are very real costs to patients when decisions take months.

- The UK is seen as 'slow' in comparison to other European countries in providing access to orphan drugs.[12]

- Up to June 2008, Single Technology Appraisals (STAs) in cancer took on average 12.8 months from the date that NICE lists in the project history to guidance date. This compares with 20.7 months for Multiple Technology Appraisals (MTAs) in cancer.[13]

- Up to December 2010, guidance from NICE took an average of 1.73 years per appraisal across all therapeutic areas. For MTA's the average is 1.97 years and STAs it is 0.96 years. On average in cancer it took 1.34 years.[14]

All of this evidence relates to the situation before the additional uncertainties of VBP and before efficiency requirements in the NHS really begin to bite. We understand that many respondents to the DH consultation on VBP highlighted their concerns on access in early 2011. The Government has partially responded by making clear that the existing funding direction for NICE positively appraised products will continue.[15] But we note that this is not an intrinsic part of VBP, but an add on which implies that the VBP approach itself is simply not sufficient to deliver on the goal of delivering access.

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[10] http://www.rarercancers.org.uk/images/stories/news/0812/The_cancer_drugs_fund_2011-12_annual.pdf
[11] http://www.rarercancers.org.uk/images/stories/news/0812/The_cancer_drugs_fund_2011-12_annual.pdf
[12] http://www.orpha.net/consor/cgi-bin/Education_AboutOrphanDrugs.php?lng=EN&stapage=ST_EDUCATION_EDUCATION_ABOUTORPHANDRUGS_EUR
[13] Barham, L Single Technology Appraisals by NICE
Are They Delivering Faster Guidance to the NHS? Pharmacoeconomics 2008; 26 (12): 1037-1043
http://www.med.mcgill.ca/epidemiology/courses/EPIB654/Summer2010/NICE/STAs.pdf
[14] O'Neil, P et al Time Trends in NICE HTA Decisions, OHE January 2012
[15] http://www.dh.gov.uk/prod_consum_dh/groups/dh_digitalassets/documents/digitalasset/dh_128404.pdf

If VBP discourages companies from launching in the UK[16], or if it is inappropriately punitive by adopting an approach not fit for assessing the full range of treatments such as those for treating rare and less common cancers, this will **make access worse not better**. If VBP results in lengthy assessment and lengthy negotiations, this will make **timely access worse, not better**. If Government fails to connect up national VBP with local decisions, **access could become more patchy across the country, not more consistent.**

B. VBP proposals could adversely affect future clinical research in the UK

Cancer52 knows that decisions to undertake clinical research in any country reflect a variety of factors and decisions of many different stakeholders: pharmaceutical companies, ethics committees, regulators, legislators (both here and internationally), clinicians and crucially patients themselves (who need to be willing to take part in trials). Such decisions are also undertaken in the broader environment that can incentivise or disincentivise innovation. Together they affect what research is undertaken, and where that research is undertaken.

There is evidence that links clinical research and local markets**.** National Economic Research Associates (NERA, 2007) found that: "In the case of clinical trials, we found that the economic arguments are clearer, with industry seeing limited value in familiarising opinion-leading clinicians with new medicines when returns in a local market are low anyway."[17]

However we note that this research was undertaken five years ago. Not only that, but the world has moved on substantially since then and policies affecting life sciences and health care systems funding have evolved. We must also take account of the impact of the global financial crisis.

A roundtable discussion event held by Myeloma UK highlighted that there are concerns about VBP and research, but also noted that not all share this concern.[18]

The broader impact on incentives for innovation, or dynamic efficiency, has been poorly covered in discussion to date on VBP. This is not just Cancer52's view, but one shared by others such as experts at the London School of Economics (LSE).[19]

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[16] The UK is but one part of the global market place: although accounting for <4% of global revenues, it is nevertheless a highly referenced country in other countries' price benchmarking (as acknowledged in OFT, The Pharmaceutical Price Regulation Scheme, 2007) and the approach to pricing and reimbursement here will affect decisions on both the launch sequence and whether to launch at all

[17] http://www.nera.com/extImage/PUB_MobileInvestments_Sep2007.pdf

[18] www.myeloma.org.uk/about-muk/news/press-releases/myeloma-uk-publishes-opinion-leaders-report-on-value-based-prici/

[19] McGuire, A et al Pricing pharmaceuticals:
Value based pricing in what sense? Eurohealth Vol 14 No 2
http://www2.lse.ac.uk/LSEHealthAndSocialCare/pdf/eurohealth/VOL14N2/McGuireRaikouandKanavos.pdf

Will VBP make the UK more attractive? Or will VBP make it even harder for the UK to be a place for clinical research? Cancer52 fears it will be the latter and not the former.

C. VBP proposals are not sufficiently clear on how they will address the specific issues that affect access to medicines for those with uncommon diseases.

The Government has not been clear about what they will or will not incorporate into the NHS treatments for those with rare and less common cancers.[20] Evidence already exists that suggests the current approaches by Government are not fit for purpose. For example, NICE recommended in 2006 that a separate committee and a higher range of Incremental Cost Effectiveness Ratios (ICERs) be adopted into rules for decision-making.[21]

Cancer52 strongly believes that international collaboration is the only way to explore the opportunities of new treatments for those with rare and less common cancers. That means that **VBP cannot simply be concerned with the UK, but must also consider the incentives for global organisations and the relative position of the UK standard of care versus other countries**.

[20] http://www.dh.gov.uk/prod_consum_dh/groups/dh_digitalassets/documents/digitalasset/dh_128404.pdf
[21] NICE, Appraising Orphan Drugs, 2006 http://www.nice.org.uk/niceMedia/pdf/smt/120705item4.pdf