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Background Paper

Assessing Value, Budget Impact
and Affordability to Inform Discussions
on Access and Reimbursement:
Principles and Practice, with Special
Reference to High Cost Technologies

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Assessing Value, Budget Impact and Affordability to Inform Discussions on Access and Reimbursement: Principles and Practice, with Special Reference to High Cost Technologies

Introduction

There are many different ways in which the value of a health technology is defined and measured. Health technology assessment (HTA) processes, often used by health care decision makers to measure and assess this value, vary around the world, combining different elements of value according to local definitions and preferences. Definitions of value generally include elements such as additional health gain and change in direct costs (and can include opportunity cost), but do not typically include a dimension relating to budget impact or affordability.

Budget impact and affordability considerations, however, may still be relevant for decision makers assigning limited resources across a health care system, and many HTA bodies and payers do take these factors into account. Recent “breakthrough” treatments (such as Sovaldi for Hepatitis C) have been shown to offer good value by most standard approaches to assessment, but have led to major challenges for affordability at the prices initially being sought by the manufacturer in a number of systems (Rosenthal and Graham, 2016; Iyengar et al, 2016), highlighting the challenge that value and affordability may not always align. Discussions at the main HTAi Policy Forum 2016 indicated that approaches to valuing innovation need to be revisited, and that questions remain as to the most appropriate role for HTA bodies (Husereau et al., 2016).

This briefing paper for the Asia Policy Forum (APF) 2016 aims to set out the issues to be addressed in tackling these challenges. The paper begins with a discussion of how value can be defined, measured and factored into decisions on access and coverage, drawing on key sources, including the discussion at the main HTAi Policy Forum in 2013 (section 1). Next, the paper looks at how budget impact and affordability can be defined and measured (section 2): we explain how different countries have adopted different approaches to how and when budget impact has been included within the decision making process, and outline several different scenarios around affordability challenges. The final section (section 3) considers whether high cost interventions call for new approaches to assessment and/or reimbursement, drawing on the recent high profile example of Sovaldi for the treatment of Hepatitis C.

1. How can we define and measure value?

Defining value

The main HTAi Policy Forum 2013 looked at how value can be measured and defined.¹ The background paper to the meeting reports the following general definitions of value:

- A fair return or equivalent in goods, services, or money for something exchanged.
- The monetary worth of something (e.g., market price).
- The relative worth, utility, or importance (e.g., a good value at the price, the value of base stealing in baseball, had something of value to say) (HTAi 2013).

However, the paper goes on to explain that there is no consensus definition of value used in health care. Discussions held at the meeting highlighted that the definition of value depends on the perspective taken: patients value improvement in length and quality of life and other improvements in the patient experience (e.g. convenience, reduction in uncertainty); the health system typically wishes to implement innovations which are of 'proven' value; the general public may wish to give increased weight to vulnerable groups (such as children or people with life-threatening illnesses); and industry generally strives to deliver value for patients, whilst maintaining commercial viability. For more information on the different perspectives see Henshall and Schuller (2013).

Furthermore, the different definitions above highlight one of the key issues when attempting to define value, which is whether or not the definition should include cost - i.e. is there a difference between *value* and *value for money*? Typically economists, and therefore HTA bodies, do include costs and/or opportunity costs in their definitions of value, i.e. value is benefit minus cost. Non-economists, however, sometimes consider value without direct reference to what something would cost them. This can potentially cause confusion when economists and non-economists come together to discuss value – as in discussions between HTA and decision makers, clinicians and patients in the health care system. To avoid confusion, throughout this paper and in the HTAi APF meeting, it is suggested that we consider that value is comprised of various elements of benefit, i.e. not including cost or opportunity cost. The terms value for money and cost-effectiveness in contrast take account of both benefits and costs, i.e. value / benefits are compared with costs.

The discussions at the meeting explored the various different value elements which can be considered. Table 1 shows an extended list of "core" and "wider" elements of value as identified at the meeting.

¹ The background paper and a report of the discussions at the meeting are both available (HTAi 2013; Henshall and Schuller, 2013).

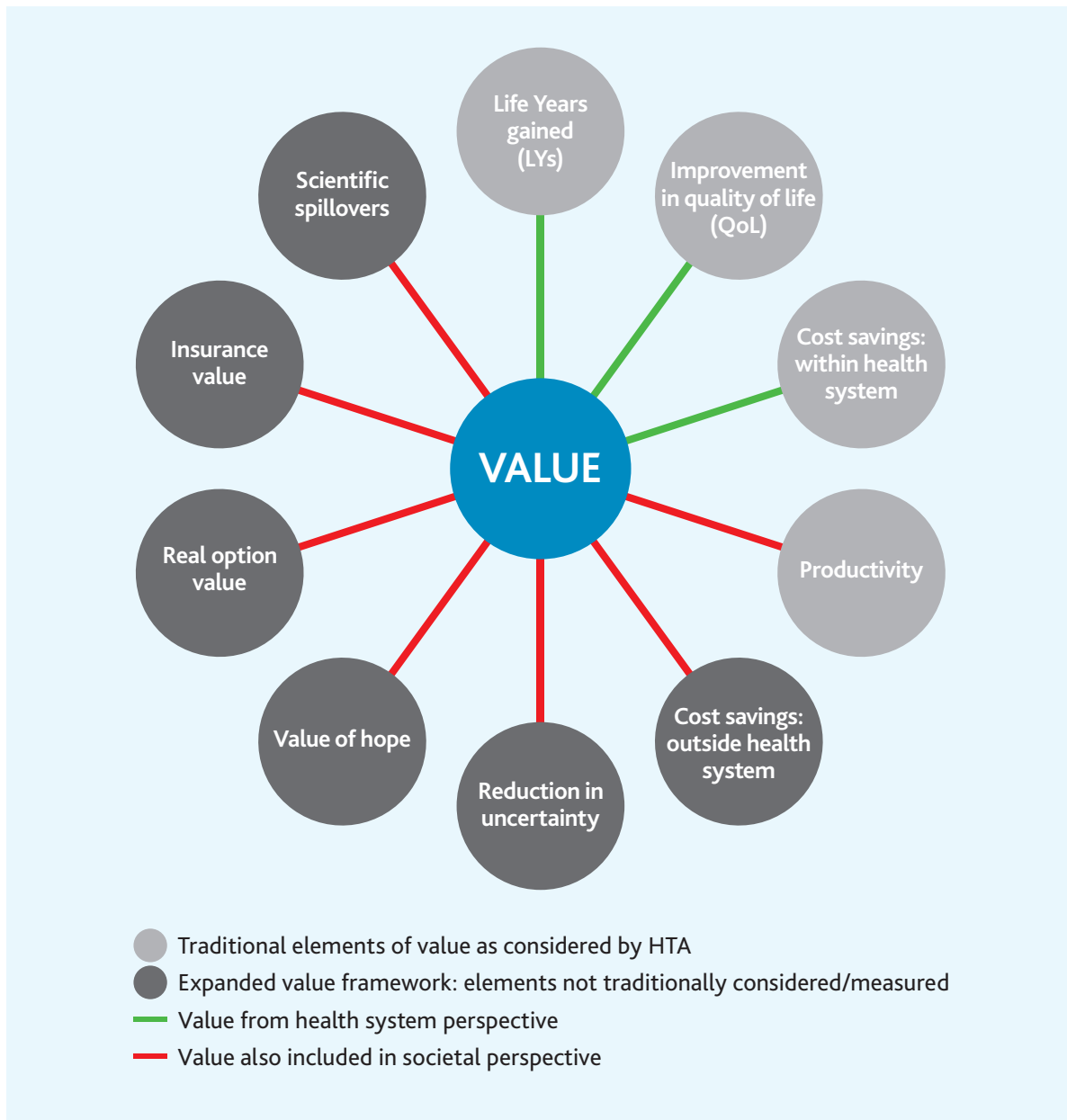
Table 1: Summary of elements of value and approaches to measurement and valuation

Elements of value	Approaches to measurement
Core elements of value	
Health benefits for the patient: <ul style="list-style-type: none"> • Improved prognosis/survival • Symptom/pain relief • Improved functioning • Reduction in adverse events 	<ul style="list-style-type: none"> • Clinical outcome measures (survival, progression of disease or disease markers, symptoms, adverse events) • Disease specific patient reported outcomes (functioning / quality of life) • Measures of overall health state, e.g., EQ5D • Composite measures of health states and life expectancy, e.g., QALY, DALY
Wider elements of value	
Non-health benefits for the patient: <ul style="list-style-type: none"> • Reduced costs • Return to work • Convenience • Reduction in uncertainty • Availability of alternatives/patient choice 	Various health economic guidelines are available for the measurement of health system costs and efficiency. In some cases there may be an indication of how other "wider" elements of value should be measured, yet there is little standardisation.
Benefits for caregiver/family. As above plus: <ul style="list-style-type: none"> • Reduced burden of care 	Measures for some elements can be adopted from areas of research such as public health (e.g., population health gain) and economic development (e.g., measures of employment and of economic benefit of innovative technology sector). Towse and Barnsley (2013) provide a discussion of various approaches to measuring such wider elements of value.
Benefits for society: <ul style="list-style-type: none"> • Support for needy/disadvantaged groups (e.g., rare diseases, diseases with high burdens, children, elderly) • Improved productivity • Economic benefits of innovation not already captured 	
Benefits for health and social care systems <ul style="list-style-type: none"> • Improved efficiency/quality/organisation of care • Changes in costs/opportunity costs • Improvements in public health and well-being 	

Source: Modified from Henshall and Schuller (2013)

Figure 1 identifies some additional elements of value identified in a recent study by OHE and EPEMED (2016). The figure includes additional wider elements of value associated with a societal perspective such as the "value of hope" (mainly relevant to an end of life situation), "real option value" (which relates to the possibility that innovations can open the pathway for other potential treatments), and "insurance value". The concept of insurance value relates to protection from health and financial risk, and therefore could be particularly important in some middle and low income countries where individuals are particularly vulnerable to health and financial shocks. The idea of insurance value has only recently been applied in work related to HTA; it is included in a new methodological development labelled "extended cost-effectiveness analysis" (ECEA) (Verguet et al., 2015a; Verguet et al., 2013; OHE and EPEMED, 2016). ECEA goes beyond traditional CEA to include the financial implications of public financing of health technologies, such as the crowding out of private expenditures which can reduce financial hardship. Such evaluation of financial impacts could be particularly beneficial in health systems that are striving to achieve universal health coverage (UHC) as UHC is concerned with not only providing services and doing so efficiently, but also protecting households from the financial risks associated with ill health.

Figure 1: Elements of value of medical technologies



Source: OHE and EPEMED (2016)

The figure also mentions scientific spillovers. This relates to how knowledge generation helps a wide group of stakeholders, not only those who produce the research. There might be economies of scope, meaning that it will be less costly or less risky to undertake further research. This is related to the economic benefits of innovation (mentioned in Table 1) which was discussed at the 2013 Policy Forum meeting. It was suggested at the meeting that there is often uncertainty at launch about the long term benefits of a new technology to patients and to the health care system. Attendees suggested that assessments could potentially consider the "promise" of an innovation as well as the value demonstrated. Promise could comprise of benefits which are likely but that have not been demonstrated fully to date; benefits from improvements in the technology; benefits which could be identified in other indications; and benefits realised through changes in the health system that arise due to the technology (Henshall and Schuller, 2013). Views differed amongst Forum members on the feasibility of such assessments, and on how they might be factored into decisions on coverage.

Many (perhaps all) HTA bodies and decision makers see patient health as the core element of value, with wider elements such as non-health benefits to the patient and their families (e.g. reduced costs, return to work), benefits for society (e.g. improved productivity) and increases in efficiency or opportunity cost considerations also playing a role in some systems (Henshall and Schuller, 2013). Towse and Barnsley (2013) echo this, suggesting that decision-makers' value determination is typically a function of benefit, cost, opportunity costs and uncertainty, but that health effect is usually the single most important element.

Systems often differ, however, in which of the wider elements of value are considered (alongside health benefit) in decision making. Indeed, Towse and Barnsley (2013) indicate that a wide range of elements of value can be identified across different HTA systems. For example, NICE in the UK always consider the health benefit to the patient and the opportunity cost of the technology; an adjustment is sometimes applied for severe or life-threatening conditions; but any changes in productivity are not considered. They adopt a health system cost and patient health perspective (as is also done in Canada, Belgium and New Zealand), whereas the French Haute Autorité de Santé (HAS) guidelines recommend taking a societal perspective (OHE and EPEMED, 2016) including additional elements of value such as changes in productivity.

Overall, it is important to recognise that the definition of value is likely to be context specific, as different health systems prioritise different elements. The relevant definition depends on the perspective which is taken, and dictates the elements of value which are included for assessment.

Measuring value

Different elements of value are measured in different ways. For health benefits at the patient level, measurement is typically quantitative through clinical trials and disease registers (although qualitative approaches such as Delphi panels may also be acceptable in some circumstances; see HTAi 2013). Measurement of changes in efficiency and opportunity cost ("economic value") is also typically quantitative, often based on cost-effectiveness analysis or therapeutic added value (see Towse and Barnsley, 2013). Many different guidelines exist globally which dictate how such clinical benefit and economic value should be measured in specific regions and circumstances. Despite some variation in methods, most health systems consider both of these value dimensions.

Measurement of the other elements of value, however, such as changes in productivity, return to work, and reductions in burden of care, can be much more difficult and methods are much less standardised (see Table 1). Measurement of improvements in productivity, for example, has been debated widely: different people contribute to the economy in different ways, and to date there is no accepted measure of these different types of contributions. Using wages as a proxy, for example, is unlikely to be an appropriate gauge amongst children, the elderly, or the unemployed.² Knies et al (2010) note that, despite many national guidelines recommending a societal perspective is taken, only South Korea and France provide detail on how to measure lost productivity.

Finally, it was noted at the APF 2015 meeting that, regardless of the decision maker's objectives at the beginning, it is likely that the only information that will be considered at the time that the decision is made is that which is available. In the context measuring value, this means that even if decision makers wish to use a definition of value that includes the wider elements of value such as productivity, problems with evidence generation (i.e. measurement of that element) may mean that it is not in practice considered within decision making.

² See Lensberg et al. (2013) for a discussion of this issue in relation to mood disorders.

Combining value elements in decision making

Once the system has determined with value elements to include and how to measure them, the next step is to assign some sort of weighting or prioritisation across the different elements of value which are being considered. Various "value frameworks", designed to help decision makers to make such decisions, are emerging. Neumann and Choen (2009) suggest the move towards such frameworks represents "a positive step". They go on to explain that by placing the focus on benefits and value, value-based approaches stimulate innovation and lead to more treatments which produce health gains, but also note that "*Value is an elusive target, and there's no consensus about what dimensions [elements] should be taken into account*". The authors review five value frameworks which have been proposed in the US, highlighting the similarities and differences between them. This review is then further built upon by Westrich (2016). The five value frameworks are:

1. The American College of Cardiology and the American Heart Association (ACC-AHA) Statement on Cost/Value Methodology in Clinical Practice Guidelines and Performance Measures;
2. A Conceptual Framework to Assess the Value of Cancer Treatment Options, developed by the American Society of Clinical Oncology (ASCO);
3. The Institute for Clinical and Economic Review (ICER) Value Assessment Framework;
4. Memorial Sloan Kettering Cancer Center's DrugAbacus;
5. The National Comprehensive Cancer Network (NCCN) Evidence Blocks.

Westrich's review (2016) looks at the framework development process, measures of benefit, measures of cost, methodology, evidence, and the framework assessment process. She explains that the five different frameworks have varying purposes, with all but one (the ICER framework) being condition specific. Of particular interest here is the "value output" which differs across each of the frameworks. Westrich summarises: The ACC-AHA assigns one of five "value levels" (high, medium, low, uncertain, not assessed) to each treatment; ASCO calculates a numerical "net health benefit score" and reports the costs separately; ICER estimates a range of prices and reports panel votes on "care value" and "provisional health system value" (either high, intermediate, low); DrugAbacus calculates a "value-based price" that represents the user's preferences and estimated costs; and NCCN presents visual "evidence blocks" including scores out of five for efficacy, safety, quality of evidence, consistency of evidence, and affordability.

Each of the frameworks includes different elements of value (all consider effectiveness and cost, sometimes aggregated and sometimes reported separately) suggesting different underlying definitions of value between the frameworks. The ICER framework includes the most complete list of elements (it allows for the inclusion of health effects, safety, patient reported outcomes (such as quality of life), indirect benefits (productivity), unmet need, burden of illness and innovation), although many of the dimensions are captured qualitatively, so, as we note above, it is not clear how they will therefore influence decision making. The review concludes by highlighting the need for good practices to guide meaningful value assessments, and notes that all health care stakeholders have the same goal of delivering high *value* to patients. Assessment of that value is challenging but will help achieve this important goal.

Typically the measurement of value elements through some sort of value framework is carried out by the HTA body (i.e. in previous APF meetings we have distinguished between the assessment and appraisal elements of HTA; this is the assessment part). Aggregation of the combined evidence across all value elements may also be undertaken by the HTA body and then passed to the decision maker for appraisal. Alternatively, aggregation of the elements of evidence of value may be undertaken by the decision maker.

2. How can we define and measure budget impact and affordability and how can these be used in prioritisation decisions?

Budget impact and affordability are not typically considered to be elements of the *value* of a technology, but can still be relevant for decision making and budget impact is often a piece of information required from manufacturers when making reimbursement submissions. Recently, several curative therapies and "game-changing" medicines have become available³ which offer good value in terms of commonly accepted value criteria. However, these valuable health gains can come at high prices, thereby posing budget challenges to health systems.

Budget impact

The budget impact of a health care technology is the total cost impact of implementing the technology (often only costs to the health care system are included) over a defined time horizon, typically only a few years. The ISPOR task force report on Budget Impact Analysis (Sullivan et al., 2014) states that: "*Budget impact analyses (BIAs) are an essential part of a comprehensive economic assessment of a health care intervention*" and states that such analyses are increasingly requested by decision makers. These analyses can help the decision makers consider the affordability of the technology and assign resources appropriately.

Budget impact analyses are useful for decision makers as budget challenges can be substantial, even when the value (however defined or measured) of a technology has been proven. However, as total budget impact is a function of treatment costs and population size, not health gain or benefit to cost ratio, there is debate over whether it is an appropriate criterion for decisions on coverage.

Affordability

Affordability concerns arise when the treatment has a high budget impact, and present particular challenges for decision makers when the treatment offers what would normally be categorised as good value at the individual patient level. In such cases, the new treatment may be of greater value than cheaper treatments which offer smaller health gains, but the health system may be unable to finance the therapies in the short or longer term, meaning that affordability issues may have to outweigh value considerations.

'Affordability' issues could refer to any of the following scenarios:

1. A technology with a substantial budget impact exposes the decision maker's doubt about the **appropriateness of the existing cost-effectiveness threshold**.
2. A technology has a **non-marginal impact** on the cost-effectiveness threshold, therefore it requires that changes be made to the decision rules.
3. Additional **time is required to adjust** to a different spending pattern – the system needs to disinvest, get efficiency improvements, or obtain higher budgets in order to fund the technology.
4. The health system wishes to **avoid paying "too much"** for the technology.
5. High up-front costs lead to problems of "cash flow", even when large savings are expected down the line. This requires **annualisation** – there is a need for a way of matching payments over the time during which benefits are realized.
6. A technology is **absolutely unaffordable** as the cost exceeds all available current and realistic potential future resourcing.

We understand that issues 3-5 are those which are most commonly being faced by health systems around the world. Potential methods to tackle affordability issues are discussed in part 3 of this paper.

³ With additional new "game-changing" medicines expected to be launched in the near future (Dennis, 2014).

The use of budget impact and affordability in prioritisation decisions

There are different ways in which budget impact could be considered within decision making, and indeed different countries have adopted different practices. Three options (note that these are general approaches not specific to when affordability becomes critical; the ways in which critical affordability issues can be tackled in the case of high cost and high value interventions are outlined in section 3 below) are:

- The **HTA body includes budget impact within its remit**. This is the case in countries such as Australia, Italy, and the Netherlands (Wilsdon and Serota, 2011), as well as Thailand (where budget impact is a recent addition to the selection criteria for the National Essential Drug List), Singapore and Vietnam (based on discussions at the APF 2015 meeting). In addition, the US based ICER group propose a framework with a price adjustment based on budget impact.
- The **payer makes decisions on affordability separately to the HTA body**. In this scenario, the price determined by HTA to represent "good value" could be considered a ceiling price, above which the payer will not be willing to reimburse the treatment. The HTA assessment is therefore the first step of a two stage process; the second step is a price negotiation. This sort of system has been used in France, Germany and Australia.
- The decision is based on **interaction between the HTA body and payers**. The HTA body could assess evidence on budget impact and other criteria (or elements of value) and passes these to the payer for decision making. This process could include a budget impact threshold, which if exceeded leads to a different payer process once the HTA decision is received, which could involve further interaction between the HTA body and payers.

Interesting, until recently, NICE in the UK only calculated total cost of a technology after the recommendation about reimbursement has been made. However, following controversy with Sovaldi, in which NICE broke convention by issuing a positive recommendation, but allowing the payer (NHS England) to delay implementation due to affordability concerns, it has recently amended its methods to state that: "*the Committee may require more robust evidence on the effectiveness and cost effectiveness of recommendations that are expected to have a substantial impact on resources*" (NICE, 2016).⁴ This implies that the committee must be increasingly certain of the value of an intervention as the budget impact increases, thereby linking the concepts of value and affordability within the decision-making framework.

Participants at the main HTAi Policy Forum 2016 discussed the relationship between value and affordability and the role of HTA in assessing these (Husereau et al., 2016). Participants commented that consideration of affordability should be included in value considerations, and that this should not be limited to searching for the lowest prices. Others stressed that affordability discussions should not be at the expense of value considerations, as this could have a negative effect on innovation and patient access to new treatments. It was suggested that enhanced horizon scanning could be useful to support upstream projection and planning.

Discussions at the APF 2015 meeting indicated that budget impact was perceived to be relevant for decision making at every level of the health care system amongst the countries represented (i.e. for national level decisions on Universal Coverage/basic package/scope of public health system; national level decisions on individual interventions/technologies; hospital level decisions). It may be particularly important for decision making at the hospital level, where there is limited access to good clinical or cost-effectiveness data. It was noted at the meeting that, whilst HTA may include budget impact considerations, in its current form it does not address affordability.

In the next section we discuss how these affordability issues could potentially be tackled. We focus on treatments variously labelled "breakthrough", "game-changing" and "curative", which might therefore be both high value and high cost, and consider whether these innovations call for new approaches.

⁴ Note that NICE and NHSE have issued a consultation paper proposing that when budget impact exceeds a ceiling a joint process involving NHSE as well as NICE should be used. <https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/consultation-on-changes-to-technology-appraisals-and-highly-specialised-technologies>

3. Do high cost medicines call for new approaches?

The affordability issues outlined in section 2 of this paper demonstrate that assessment of value is insufficient for allocating resources within a health care system: treatments can be high value, cost-effective, and yet unaffordable. The fundamental issue of *paying for* these high cost interventions has not, to date, been adequately addressed.

How have we managed so far?

In Europe the approach taken to Sovaldi (sofosbuvir)⁵ and Harvoni (a combination of sofosbuvir and ledipasvir) was to “muddle through”, largely with aggressive discounting, restricting access, and budget capping with rebates, the consequences of which have not necessarily been fully thought through (the potential adverse effects of this type of mechanism are outlined below). In the UK, for example, as noted above, NICE found the drugs to be cost-effective (at least in some population groups); but, based on affordability concerns, NHSE (the payer) delayed implementation for four months in order to find the resources and create the infrastructure to support use of the treatment. This had never been done before and innovators were unhappy with this new unexpected hurdle. In the US, payers quickly began using competing new drugs to Harvoni to obtain large discounts from all Hep C drugs. Some payers (for example the US Department of Veterans' Affairs) also restricted access to a certain patient sub-groups in order to mitigate budget impact (Husereau et al., 2016). In Australia, an innovative risk-sharing approach was implemented whereby fixed reimbursement was agreed over a set period time to mitigate high upfront costs.

Clearly, comprehensive and transparent processes which guide how affordability, as well as value, will be assessed, need to be put in place. These processes need to provide clear instruction on how decision making takes both of these factors into account, and explain how the decision maker will respond if a high value treatment leads to a potential affordability challenge.

Mechanisms to manage affordability

There are several mechanisms available which could be used to mitigate affordability concerns:

- **Discounts and revenue caps** (as has been seen with Hepatitis C around the world): There are two aspects to this. One is general bargaining to reduce the initial price offered by the innovator, the other is more interventionist – questioning what return a manufacturer can and should make. Such a motivation would cap returns on R&D to “reasonable” or “affordable” levels to avoid paying “too much” for the therapy. Whatever the motivation, these mechanisms restrict spending, and therefore control budget impact. However, they may send the wrong signals to industry about the importance of R&D. For example, using similar maximum revenue caps across different diseases would send the signal that payers are indifferent between them, which may not be the case. Clearly incentives to find additional cures will be strongest when the innovator receives a higher share of social return within the patent period.
- **Targeting the highest value patient groups** until additional money is available to extend to other patient groups, thus “buying time” for the payer to adjust to changes in spending. This has been seen in practice in England where the implementation of Sovaldi was delayed, and France, Spain and Italy where use of the new medicines was restricted to patients with cirrhosis of the liver (i.e. those most in need) in order to limit uptake. Problems remain, however, as perverse signals are sent to innovators in the short run, and patients who can benefit from a curative therapy are likely to resist such rationing of access.

⁵ Sovaldi was only the third drug to receive the “Breakthrough Therapy” designation by the FDA. It is a highly effective treatment for hepatitis C, with trials demonstrating that it can effectively cure 90 percent of people with the virus (Schiff, 2015). However, costs are high: at the list price and recommended dose, a course of treatment costs around US\$84,000 per patient (Humer and Beasley, 2014).

- **Managed entry agreements** (also called risk-sharing agreements, coverage with evidence development, etc.): Forcier and Noël (2013) describe two categories of financial-based agreements that have been used to improve affordability across various markets: 1) rebate agreements, where a reduced confidential price is offered to the payer, and 2) price-volume agreements, in which the price is reduced according to utilisation of the therapy, thereby effectively capping budget impact. These approaches can help control spending, and have been used fairly widely, for example Patient Access Schemes in the UK and payback mechanisms in France. Yet, they are imperfect, as there is a lack of transparency over the price that is being paid (usually only list prices are publically available), and more complex agreements can be administratively burdensome for health systems that are already stretched.
- **Pay-for-performance:** This type of mechanism could be considered to be a hybrid between two others: managed entry agreements and annualisation (discussed below). This sort of agreement leads to the manufacturer receiving payment per unit of health, for example, per year that a patient remains free of disease. This combines risk sharing, as the payer only pays for positive results, and annualisation, as the high upfront cost is spread out over time.
- **Annualisation:** This involves using payment models that spread the potentially high upfront costs across the duration that the benefits will be realised. This is linked to the idea that new treatments provide a life time of health gain for patients and thus may be cost-saving for health systems in the long term, but high upfront costs means that the costs are accrued long before the benefits are realised (see affordability issue 5, section 2). A recent OHE literature review (Karlsberg Schaffer et al., forthcoming) found that the most commonly proposed solution to affordability problems is to spread high upfront costs over a longer period of time, i.e. the manufacturer is paid in instalments. Gottlieb and Carino (2014) argue that mechanisms such as these help to align the cost of the cure with its long-term economic benefits, thereby allowing payers to fund the treatments whilst balancing their budgets within a single year. They note that such agreements are already common with medical equipment, with which payers often spread the cost over the time horizon that the equipment will be used.
- **Credit market solutions:** These can be considered to be similar to mortgages or loans, whereby the government (or another third party) issues loans to payers to fund the upfront bill, and then the health care payer pays instalments over time, in line with realisation of the benefits (Philipson and von Eschenbach, 2014)⁶. An example of this type of mechanism can be seen in Spain, where the national government announced low-interest loans for regional payers to fund high cost Hepatitis C therapies (APMHealthEurope, 2015).

This is not considered to be an exhaustive list of possible mechanisms, and in fact the different options could also be used in combination (for example in Spain the loans will be used in combination with managed entry agreements; APMHealthEurope, 2015).

Other 'problem' areas

Throughout the preceding examples we have concentrated on high cost curative therapies, but it is worth acknowledging that health systems could also face similar problems in association with gene therapy, other regenerative medicines and orphan drugs. Orphan drugs in particular can be problematic given small patient numbers and high uncertainty around value for each individual drug, with multiple orphan drugs leading on aggregate to a high budget impact. Many systems have different criteria or reimbursement arrangements in place for orphan drugs, and perhaps need to consider whether alternative arrangement are also required in other areas.

⁶ Note that this is a variation on the annualisation approach described above.

Summary

In summary, definitions of the value of a health technology vary around the globe, as demonstrated by the inclusion of different value elements within value assessments. Typically health benefits to the patient are considered to be the most important value element, with cost-effectiveness or therapeutic added value also being considered by many HTA bodies and decision makers. Ultimately, whatever value elements are to be considered, they must be measurable (either qualitatively or quantitatively); in practice decision makers are likely to only consider elements for which evidence is available at the time that the decision is made.

Budget impact and affordability are not generally considered to be value elements, but are still often relevant to decision making, as establishing that a technology is high value in terms of clinical and cost effectiveness does not automatically signify that it is affordable or the correct priority for a cash-limited system, at least in the short run (as demonstrated by the example of Sovaldi). Countries around the world have differed in their approach to assessment of budget impact and affordability, with some including it in the HTA process (either directly or via use of a measure of opportunity cost in the HTA process as the proxy for affordability), and others treating it as a separate analysis outside of priority setting. Health systems and payers have “muddled through” with high cost interventions to date, but it is apparent that processes and methods are required to provide transparent guidelines in situations where affordability concerns seem to threaten the availability of high value treatments.

Several mechanisms are available to mitigate affordability concerns, some of which have already been trialled on an ad hoc basis in relation to Sovaldi. Governments, health systems and payers need to be aware, however, of any unintended adverse incentives on innovators, particularly at a time when rapid advances are being made in basic and clinical science (for example gene therapy) and further major advances may soon be feasible.

This briefing paper for the APF 2016 has set out the issues to be addressed in tackling current challenges in value assessment and affordability. The issues will be further discussed, examined and refined throughout the course of the meeting.

References

- APMHealthEurope, 2015. Prices of new hepatitis C combinations lower than monotherapy in Spain in six months. Available at http://www.apmhealthurope.com/Prices-of-new-hepatitis-C-combinations-lower-than-monotherapy-in-Spain-in-six-months-FS_41696.html [Accessed September 2016].
- Dennis, B. 2014. *Drugmakers find breakthroughs in medicine tailored to individuals' genetic makeups*. Available at: http://www.washingtonpost.com/national/health-science/drugmakers-find-breakthroughs-in-medicine-tailored-to-individuals-genetic-makeups/2014/06/01/40127d1c-e107-11e3-8dcc-d6b7fede081a_story.html [Accessed August 2016].
- Forcier, M.B. and Noël, F., 2013. Product listing agreements (PLAs): a new tool for reaching Quebec's pharmaceutical policy objectives?. *Healthcare Policy*, 9(1), p.65.
- Gottlieb, S., & Carino, T. 2014. Establishing new payment provisions for the high cost of curing disease. *AEI Research*.
- Henshall, C., Schuller, T., 2013. Health technology assessment, value based decision making, and innovation. *Int J Technol Assess Health Care*, 29(4), pp.3539.
- HTAi, 2013. *HTA and Value: Assessing Value, Making Value-Based Decisions, and Sustaining Innovation*. Background paper to the HTAi Policy Forum Meeting 2013.
- Humer, C., and Beasley, D. 2014. *Insurers scrutinize drug costs after \$84,000 Sovaldi surprise*. Available at: <http://www.reuters.com/article/us-usa-healthcare-hepatitisc-insight-idUSKBN0E80AZ20140528> [Accessed August 2016].
- Husereau, D., Sampietro-Colom, L., Henshall, C., Thomas, S., 2016. Changing Health Technology Assessment Paradigms? *Int J Technol Assess Health Care*, 32(4), pp.1.
- Iyengar, S., Tay-Teo, K., Vogler, S., Beyer, P., Wiktor, S., de Joncheere, K., Hill, S., 2016. Prices, Costs, and Affordability of New Medicines for Hepatitis C in 30 Countries: An Economic Analysis. *PLoS Med*, 13 (5).
- Karlsberg Schaffer, S., Messner, D., Tambor, E., Towse, A., forthcoming. *Paying for Cures: Perspectives on solutions to the "affordability issue"*. OHE Research.
- Knies, S., J. L. Severens, A. J. Ament, and S. M. Evers, 2010. The transferability of valuing lost productivity across jurisdictions. Differences between national pharmacoeconomic guidelines, *Value in Health* 13(5), pp. 519-527.
- Lensberg, B., Drummond, M., Danchenko, N., Despiegel, N., Francois, C., 2013. Challenges in measuring and valuing productivity costs, and their relevance in mood disorders. *Clinicoecon Outcomes Res.*, 5, pp.565-573.
- NICE, 2016. *Developing NICE guidelines*. NICE Process and methods, PMG20. London: National Institute for Health and Care Excellence.
- Neumann, P. and Cohen, J., 2009. Measuring the Value of Prescription Drugs. *The New England Journal of Medicine*, 373, 27.
- OHE and EPAMED, 2016. *The Value of Knowing and Knowing the Value: Improving the Health Technology Assessment of Complementary Diagnostics*. White paper. Available at: http://www.ohe.org/sites/default/files/WP_EpemedOHE_final.pdf [Accessed September 2016].
- Rosenthal, E and Graham, C., 2016. Price and affordability of direct-acting antiviral regimens for hepatitis C virus in the United States. *Infectious Agents and Cancer*, 11, 24.
- Schiff, L., 2015. Finding truth in a world full of spin: myth-busting in the case of Sovaldi. *Clinical therapeutics*, 37(5), pp.1092-1112.
- Sullivan, S., Mausekopf, J., Augustovski, F., Caro, J., Lee, K., Michin, M., Orlewska, E., Penna, P., Barrios, J., Shau, W., 2014. Budget Impact Analysis—Principles of Good Practice: Report of the ISPOR 2012 Budget Impact Analysis Good Practice II Task Force. *Value in Health*, 17, pp.5-14.
- Verguet, S., R. Laxminarayan, and D. T. Jamison, 2015a. Universal public finance of tuberculosis treatment in India: an extended cost-effectiveness analysis. *Health Economics*, 24(3), pp. 318-332.
- Verguet, S., Olson, Z., Babigumira, J., Desalegn, D., Johansson, K., Kruk, M., Levin, C., Nugent, R., Pecenka, C., Shime, M., Memirie, S., Watkins, D., Jamison, D., 2015b. Health gains and financial risk protection afforded by public financing of selected interventions in Ethiopia: an extended cost-effectiveness analysis. *The Lancet Global Health*, 3(5), e288-296.
- Verguet, S., S. Murphy, B. Anderson, K. A. Johansson, R. Glass, and R. Rheingans, 2013. Public finance of rotavirus vaccination in India and Ethiopia: an extended cost-effectiveness analysis. *Vaccine* 31(42), pp. 4902-4910.
- Wilsdon, T. and Serota, A., 2011. *A comparative analysis of the role and impact of Health Technology Assessment*. CRA Project No. D15891-00 for EFPIA, PhRMA, Medicines Australia and EuropaBio. London: Charles River Associates.



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