Outcomes-based reimbursement of medicines

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Social Market Foundation

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EXECUTIVE SUMMARY

This report describes the benefits of introducing an outcomes-based model for drug purchasing as a mechanism to address the affordability challenge, to reduce risks within the NHS and to improve outcomes for patients. This would apply the principle of the NHS only paying for what works. The report describes how such a scheme could be introduced and the design challenges to be overcome.

The NHS is facing huge challenges on three fronts: its budget is constrained, with per capita spending on healthcare falling; demand is growing as a consequence of demographic pressures and rising co-morbidities; and, the reform agenda towards personalised, outcomes-based care is gathering pace. Transforming how care is commissioned and provided, whilst delivering better value for money are the over-riding purposes as the NHS pursues efficiencies of £22bn over five years.

Yet, how we procure medicines is too often left out of the equation. This is despite the £16.8 billion that is spent each year on treatments, the importance that patients attach to prompt access to effective treatments and the opportunities for innovation. Reform-orientated initiatives such as the Cancer Drugs Fund and the Accelerated Access Review have pointed towards a path of change, but the principal policy reaction has been one of cost control. Pro-innovation industrial policy that seeks to maximise the contribution of the Life Sciences sector is pitted against healthcare policies designed to meet funding constraints. Continue to follow this road and we sacrifice long-term value for short-term control. This will harm patient outcomes and damage the UK economy, undermining investment in the health economy which accounts for a fifth of R&D spending. Instead, the NHS and medicines procurement must re-focus on value for money over the longer-term.

To this end, this paper assesses the potential benefits of introducing outcomes-based reimbursement for drugs as an alternative approach to funding medicines and sets out how it could be pursued. We conclude that this could be an important reform for the new Government to pursue as it takes forward recommendations through the Accelerated Access Review.

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Why we should introduce outcomes-based reimbursement

The access to new medicines in the UK contains features that may negatively affect patients, the efficiency of the NHS and the wider economy. As this paper argues, adopting an outcomes-based commissioning model could result in improved patient outcomes: contributing to earlier access to medicines, driving better value for money and reducing the risks for the NHS, facilitating more personalised prescribing and offering clearer incentives to invest in the UK's health economy and in innovative treatments. In some specific instances, this approach could also provide the infrastructure for the NHS to fund medicines over multiple years thus reducing the burden within a specific financial year.

Our research learns from similar developments in other countries as well as application in UK public services. For instance, managed entry agreements in Italy have similar features to the outcomes-based reimbursement model discussed in this report. Two of the schemes require the attainment of pre-determined outcomes. Under Payments-by-Results, the manufacturer must pay back the full amount paid for the medication for each non-responder to the Italian commissioner. Under Risk Sharing contracts, the manufacturer must pay back a proportion of the amount based on non-responders. As of October 2012, there were 16 Payments-by-Results contracts in place in comparison to two Risk Sharing contracts. In the UK, outcomes-based approaches have been adopted in employment support and offender rehabilitation, whilst they are being pursued actively in the NHS and social care. The report discusses the options and trade-offs when determining the outcomes that should be pursued, including putting a value on 'unmet need' and potentially other wider economic and social outcomes, whilst not reducing health decisions to mere fiscal equations.

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How we should introduce an outcomes-based scheme

The policy proposals set out in this paper seek to build on the successes of our current infrastructure and institutions, as well as the Accelerated Access Review. Specifically, the proposals cover:

- Inclusion criteria: Terms for deciding what new treatments should be pursued through the scheme. One such condition that we propose is that drugs that address 'unmet' need could progress into the scheme.
- Definition of outcomes: With the NHS to agree the outcomes that the drugs should achieve in order to be remunerated, applying the principle of only paying for what works.
- Better measurement and data: An outcomes scheme would be reliant on good measurement and data. Further work is needed to build momentum behind initiatives to develop better data on healthcare outcomes and the necessary architecture. This would be of wider value to commissioners, patients and policymakers across the healthcare economy.
- Cost sharing options: Enabling an option for the manufacturer and the NHS to share the initial costs of interventions, thus allowing the NHS to spread the costs of treating large cohorts of patients over multiple years.

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INTRODUCTION

This research assesses the potential benefits of introducing outcomes-based reimbursement for drugs as an additional route for bringing medicines to market in the NHS and explores how such a scheme should be designed.

It seeks to answer the following questions:

- What are the problems with how the NHS purchases drugs currently?
- What would be the potential benefits of moving to a more flexible system enabling an outcome-based approach?
- What are the main considerations in designing an outcomes-based scheme?
- What could the decision-making process look like?
- How could we overcome and manage specific design challenges?

It starts, in Part I, by assessing the flaws in the UK's current ability to access new and novel medicines. Part II sets out the potential benefits in introducing a scheme with outcomes-based payments for drugs. Part III analyses how best to design a viable scheme, including what outcomes to pay for, how outcome risk should be shared between the NHS and the manufacturer and who pays and when. Part IV provides an outline of the commissioning process and Part V describes how the wider system would need to change to facilitate and incorporate such schemes efficiently as a route to improving patient outcomes.

Our analysis was based on a wide review of the literature and evidence relating to UK and European healthcare, and to drug reimbursement schemes in the UK and abroad. Our evidence review also covers payment by results schemes and contracting adopted more widely in UK public services. The research was informed by discussions with experts, officials, patient charities and industry.

PART I: HOW DO WE REIMBURSE DRUGS CURRENTLY AND WHAT ARE THE CHALLENGES?

This section describes how drugs are currently reimbursed and explains why we need reform.

What society wants from a well-functioning purchasing model

The NHS is witnessing one of the most prolonged periods of budget constraint since its inception. Real-terms increases of around 1% per annum this decade compare to an historical average of 4%. Per capita spending and spending as a proportion of GDP are both in decline.[1]

In this context, continuing to maximise value for money is paramount, including in the £16.8 billion spent on medicines in 2015/16.[2] Value for money derives from the benefit delivered to the patient and society versus the costs to the purchaser. This must underpin any reimbursement model – and NICE technology appraisal comprises a rigorous process to assess cost effectiveness. But, more broadly, our analysis leads us to believe that a well-designed reimbursement model should fulfil several critical functions:

- It should ensure that patients are able to access new and innovative medicines as early as possible whilst facilitating good value for money for the NHS and hence the taxpayer.
- It should ensure that the value created by treatments to patients and society is captured and rewarded so that the pharmaceutical sector is encouraged to develop treatments that are of the greatest value to society.
- It should ensure that investors receive clear signals to develop and introduce new treatments by ensuring that there is a strong and clear incentive to invest.
- It should fit properly within a wider outcomes-focused NHS so that commissioners are able to choose the optimal combination of interventions through the care pathway whether that is primary, secondary or community care, social care, medical devices or medicines.

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How drugs are currently made available

The NHS has a mechanism to regulate medicines available to patients but it can be disjointed in its approach. In a scenario where individuals' health risks are insured by the state, prescribers and patients – who make the ultimate decision on whether a drug is used – may be unaware of the price of drugs they consume (or prescribe), and may be insufficiently sensitive to the price of drugs.[3] Change this sentence to: A framework agreement is in place to ensure pricing is managed and help facilitate appropriate pricing.

Current regulations within the healthcare industry centre on the Pharmaceutical Price Regulation Scheme (PPRS). Introduced in 1957, the PPRS is a voluntary agreement between the Government and the pharmaceutical industry. The current scheme runs for five years from 2014, and it covers branded medicines supplied to the NHS by scheme members, regardless of their patent status. The scheme has historically regulated the profits that companies can achieve on sales to the NHS. It is based on a range of maximum allowances covering R&D, manufacturing costs, information, sales and marketing and general administrative costs. These are then subject to a maximum percentage profit. Overall, the scheme aims to manage the need for new medicines in the future and the government's desire to control expenditure. Due to economic conditions and the financial challenges facing the NHS, the Association of British Pharmaceutical Industry (ABPI) agreed with the Department of Health in 2014 for the current scheme, as an exceptional measure, to limit the growth in the overall cost of branded medicines purchased by the NHS from members of the scheme. This is delivered by a cap and levy whereby companies repay any growth in expenditure beyond agreed levels (in the range 0 -2% in 2014-2018) to the Department for Health.

New medicines may also require an evaluation of clinical and cost effectiveness carried out by the National Institute for Health and Care Excellence (NICE). NICE evaluates the drug in terms of extension of life and quality of life improvements and this is set against the cost. NICE will recommend medicines where the cost of a Quality Adjusted Life Year (QALY) is below cost-effectiveness thresholds.[4]

The shortfalls in our current system

Notwithstanding the fact that the UK has well-established and respected decision-making institutions and infrastructure, the current system has a number of drawbacks.

1. Delays in access to new treatments and questions over value for money

In some instances our current system can delay patient access to new treatments. So as to be able to estimate the potential effectiveness of the drugs more precisely, evidence gathering to inform pricing and reimbursement is carried out ex-ante before the drugs are made available to patients. This is time-consuming. The scale of the delay is illustrated by a claim by PWC's Strategy & team in its paper for the Accelerated Access Review (AAR) that if their recommendations were to be implemented patients would be able to access medicines up to six years earlier after marketing authorisation.[5] Patients and the charities representing them are frustrated by the level of access to the newest and most effective treatments.[6]

This dilemma is particularly marked in relation to drugs that are highly innovative – because the outcomes are harder to predict and there could be uncertainty surrounding the clinical data, commissioners may decide that the risks are too great to make the drug available.

The Coalition Government created alternative pathways for more rapid drug access, including the Early Access to Medicines Scheme and the Cancer Drugs Fund. However, balancing the desire to make drugs available to patients promptly with the requisite focus on value for money has proved challenging.

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Box 1: Patient Access Scheme, The Early Access to Medicines Scheme and the Cancer Drugs Fund

Patient Access Scheme:

Patient Access Schemes (PASs) were introduced in the 2009 PPRS. The schemes are designed to facilitate patient access to a medicine where the list price is higher than the value derived by NICE during their assessment. The agreements are made between the Department of Health and the pharmaceutical company, with involvement from NICE. There are two types of schemes: simple discount schemes and complex schemes. Simple discount schemes should ensure no significant ongoing burden for the NHS, and a manufacturer can reduce the price of the product. Complex schemes involve more intricate agreements; these can include outcome-based schemes, dose capping and stock supplied at zero cost.[7]

There is currently a preference by the Department of Health for simple discount schemes. According to NICE data, there have been 113 PASs between 2007 and 2017.[8] A significant proportion of these have been simple discount schemes, whilst only one has been based on outcomes. The burden of data collection and the absence of data standards may make the commissioner reluctant to participate in complex schemes, although other factors may play a part.

Early Access to Medicines Scheme:

The Early Access to Medicines Scheme (EAMS) was set up in April 2014 with the aim of providing earlier availability of promising new unlicensed medicines (medicines that do not have a marketing authorisation) to UK patients that have a high unmet clinical need.[9] The medicines included in the scheme are those that are intended to treat, diagnose or prevent seriously debilitating or life threatening conditions where there are no adequate treatment options. Under the scheme, the Medicines and Healthcare products Regulatory Agency (MHRA) gives a scientific opinion on the benefit/risk balance of the medicine, based on the data available when the EAMS submission was made. The scheme has resulted in a number of drugs being available to patients a number of months before they received marketing authorisation.

Cancer Drugs Fund:

In 2011, the government established the Cancer Drugs Fund (CDF). It was introduced to help support patients gain access to cancer drugs not routinely available on the NHS. A new framework for CDF has come into force to address perceived unsustainable financial pressures. As part of this new framework, NICE began reappraising all old drugs within the CDF in April 2016. Drugs have one of three fates: rejected, stay in the CDF, or recommended for routine commissioning.

NICE can make the recommendation that a drug should enter the CDF when "there is potential for a drug to satisfy the criteria for routine commissioning, but where there is currently too much uncertainty surrounding the clinical data and consequently the cost effectiveness estimates to make such a recommendation".[10]

The CDF has enabled patients to access drugs – such as sorafenib for treating advanced hepatocellular carcinoma – that would otherwise have been unavailable. However, recent academic research has suggested that previous CDF practices were unlikely to achieve value for money.[11] The study focuses on the potential benefits associated with 47 indications that were available through the CDF in January 2015. This analysis relates to the original CDF, which has been replaced by a new CDF that acts as a managed access fund.

While these interventions have had some success in enabling patients to access some drugs that would otherwise would not have been available, they have limitations. Indeed, even in 2010, the Government admitted that the CDF and the PAS were 'not long-term solutions'.[12] Since inception, over 95,000 patients have benefited from the CDF.[13] However, the fund is limited to cancer drugs. More fundamentally, concerns have been raised that drugs made available through the initial CDF were poor value for money (see Box 1 above). The lack of connection between the amount paid for the drugs and the benefits to patients has led to significant pressure to constrain spending through the CDF. To the extent that the CDF has resolved the problem of delayed access, it has done so by exposing the taxpayer to higher risks than would occur through an outcomes-based approach. Changes need to be made so that patient access issues are addressed whilst retaining value for money.

The EAMS on the other hand is open to treatments for all diseases but fewer non oncology drugs have been made available through this route.[14] While the PAS route potentially adds significant flexibility into the system, few drugs have been approved using an outcomes-based approach.

This is symptomatic of a wider flaw within the current model, namely that manufacturer's remuneration is not connected sufficiently with the real life performance of their products. Estimates produced through trials under controlled conditions may be an inaccurate reflection of their impact on wider patient groups and at different dosage levels. Moreover, as the Office of Fair Trading pointed out a decade ago, drugs with the same properties may be purchased at very different prices, though the practice of prescribing generic equivalents has increased significantly over time.[15]

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2. Improving investment incentives for investors in the health economy

A second problem is that a system of profit and revenue claw-backs limits the reward that investors can receive. Providing clearer incentives could help attract additional investment into the UK's health economy and to develop new treatments for patients.

The UK's Life Sciences and wider health economy is a competitive strength of the UK. Data from the ONS shows that 'Medicinal and Pharmaceutical products' are the third largest category of UK goods exports, constituting 8.5% of the value of all UK exports. As Figure 1 shows, in 2015, the pharmaceutical industry accounted for around a fifth of UK R&D spending. This investment contributes not only to UK growth, jobs and exports but also to the availability of new treatments for patients. As part of the Industrial Strategy, Sir John Bell was invited to develop a Life Sciences economic strategy to identify steps to advance the UK's competitive strengths, recognising the value of a thriving life sciences sector to the UK economy. This builds on significant work developed through the Accelerated Access Review.

Recent independent research produced by KPMG Economics team and commissioned by the National Institute for Health Research (NIHR) Clinical Research Network estimated the economic contribution made to the UK clinical research activity supported by the network at £2.4 billion of gross value added.[16]

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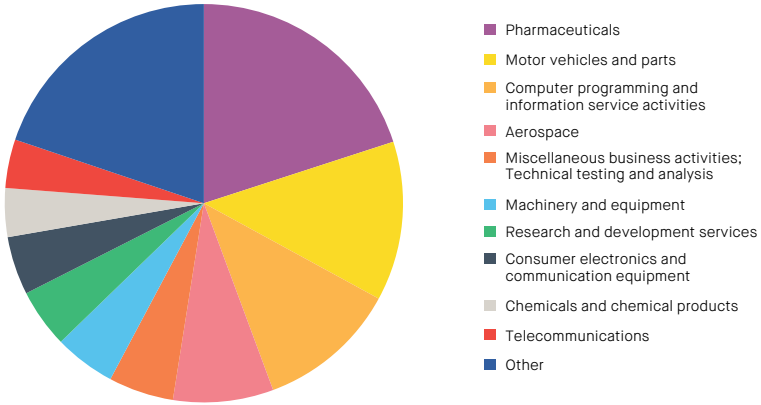
Figure 1: Expenditure by UK businesses on performing R&D in 2015 (2015 prices)



Source: ONS, Business enterprise research and development, UK: 2015 (2016)

However, our current systems for reimbursing and making treatments could be re-designed to improve the incentives for productive investment.

Clawing back profits reduces the incentives for firms to control costs. It may lead to comparatively low incentives to invest in high risk or innovative interventions compared to lower risk interventions because the upside is capped.[17] Successful firms that innovate at a lower cost than others do not reap the rewards.

Second, the healthcare system operates on a presumption that there is a fixed sum that should be spent on a medicine (as compared implicitly to other health service interventions). Setting quotas for how money is spent within the system is inefficient and reduces the scope for innovation. Over time, blunter tools have been added as cost control has trumped value for money and innovation. The Health Service Medical Supplies (Costs) Act indicates a path. As of 1 April 2017, NICE and NHSE has implemented a budget impact test. Any new medicine that passes NICE's cost effectiveness evaluation will also be subject to a commercial negotiation if the total net budget impact exceeds £20m in any of the first three fiscal years post launch.[18] This injects further uncertainty for investors.

The consequence is that investors have little confidence that important new treatments they discover that address unmet need will be approved and purchased; they also have no confidence of the price that will be paid irrespective of its value; and they have little incentive to maximise innovation because more profitable ventures do not pay higher returns. Meanwhile, patients are denied treatments that are available and that have in some cases passed NICE cost effectiveness tests.

The NHS is one of the largest publicly-funded healthcare systems in the world and is the main customer of medicines in the UK (with only around one in ten of the UK population having some form of private medical cover).[19] The Government has a huge strategic opportunity to promote the conditions for innovation, investment and competition. In its consultation, the Department for Business, Energy and Industrial Strategy was right to ask: 'What further steps can be taken to use public procurement to drive the industrial strategy in areas where government is the main client, such as healthcare?' As described below, one of the answers lies in re-thinking our reimbursement model.

3. Sharpening the focus on innovation and capturing broader value

A further dilemma in our current procurement model is that our calculation of what matters – encapsulated in NICE's definition of a QALY – arguably provides an incomplete measure of what matters to society. A QALY is a measurement of improved health; this form of measurement is used within a number of OECD countries. QALYs are calculated by estimating the years of life remaining for a patient following a particular treatment or intervention and weighting each year with a quality-of-life score (on a 0 to 1 scale). Measures include the person's ability to carry out the activities of daily life, and freedom from pain and mental disturbance.[20] NICE will recommend drugs with a QALY of between £20,000 and £30,000 and up to £50,000 per QALY for end of life care drugs.[21]

QALY is respected as a robust methodology. However, there are broader dimensions of value that society may wish to reward and encourage. First, society may want to pay more for new innovative developments including

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treatments that meet 'unmet' need so as to send a signal to investors to develop such treatments. Second, society may wish to put a greater weight on outcomes for some specific patient groups – for example, for patients with severe, chronic or terminal diseases.[22] Third, society may wish to pay for a wider suite of outcomes. These could include fiscal and economic benefits, and morbidity as well as mortality measures.

4. Catching up with reforms in wider healthcare system

In the context of increasing co-morbidities, extended lives and rising costs of healthcare, there is growing interest across the wider NHS in personalised care, value-based healthcare and outcome-based commissioning. Sustainability Transformation Plans set out ambitions to transition from procuring on the volume of care activities (e.g. hospital appointments or operations) to commissioning on the basis of outcomes.[23] As the Bedfordshire, Luton and Milton Keynes Sustainability and Transformation Plan states: 'Commissioners will become more focused on the health and wellbeing of local people and on clinical outcomes where services are provided, rather than inputs and processes.'[24] Some STPs include new commissioning models which 'share risk between organisations, focus on outcomes of care', for instance through capitation payments, where a provider is given payment to reflect the needs of the population and left with the responsibility to improve healthcare outcomes.[25]

Moving to an outcomes-based healthcare system facilitates a profound shift. As Michael Porter has argued, 'We must move away from a supply-driven health care system organized around what physicians do and toward a patient-centered system organized around what patients need'.[26] Such an approach means commissioning the optimal mix of care options to deliver value and achieve outcomes (whether via hospital, social care, medicine or community care). The move to a single commissioner at the sub-regional level aims to facilitate this. But, drug treatments are not part of this continuum because methods for reimbursing them are out of sync with where the wider healthcare system is moving.

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PART II: WHAT WOULD BE THE POTENTIAL BENEFITS OF ENABLING AN OUTCOME-BASED APPROACH?

Part I described a number of shortfalls in the current system. Many of these could be addressed by applying the concept of 'outcomes-based' commissioning to the way in which medicines are used in the real world.

Potential benefits of moving to an outcomes-based approach

The aim of an outcome-based scheme is to link the remuneration that firms receive to the actual performance of their product, in terms of the real benefits that it brings to the patient and wider society.[27] In so doing, such an approach has a number of particular attractions:

- Only paying for what works: Currently, where medications do not yield results as expected the NHS achieves poor value for money. Under an outcomes-based scheme, the amount that the drug company receives in the long-run would be dependent on the treatment conferring a predetermined level of benefit. Academics who recently criticised the old CDF scheme for achieving poor value for money suggest that 'improvements on the current system could also be achieved through new payment systems based on the attainment of pre-determined outcomes or the introduction of value-based co-payments.'[28]
- Innovative therapies could be made available to patients earlier: Schemes such as the CDF and EAMS are already allowing earlier access to certain medications. By protecting the NHS from the risks of underperformance of treatments where outcomes are uncertain, the NHS could permit earlier local access to drugs with greater confidence through an outcomes-based approach.
- Aligning the incentives for investment in R&D with the needs of NHS patients: Major drug discoveries that improve health would receive more generous reimbursement, whilst the NHS budget would be protected from paying for treatments that are not sufficiently effective.[29]

- Aligning the interests of manufacturers and commissioners thus permitting better targeting of drugs within the patient population. Under an outcomes-based scheme, the manufacturer only has an incentive to recommend that a patient receives a drug where there is a reasonable likelihood that the patient's health will improve. Therefore, the interests of both the NHS and the manufacturer are aligned in favour of appropriate patient selection. According to a study analysing the use of atorvastatin in an outcomes-based reimbursement scheme found that by making the manufacturer accountable for outcomes it was more likely that the product would be targeted at those patients who would benefit the most.[30]
- Transforming innovation in treatments with wider reform of the health service. Adopting an outcomes focus would support the wider healthcare transformation programme as any system improvements required to drive patient outcomes could form part of the overall agreement. The approach could bring two associated benefits: it would shine a light on the performance of therapies in standard clinical practice (rather than within a trial); and, it would also drive a greater focus on outcomes data in the NHS.
- Assisting the NHS to manage its short and medium term budget constraint challenges. As will be described below, by sharing the risk, outcomes-based agreements could help alleviate cost pressures in the medicine budget, especially if such agreements spanned over several financial years.

Building on current reforms

There is fertile territory to develop this concept. As noted in the Five Year Forward View and subsequent strategies, NHS England and the Government recognise the importance of innovative reforms, including outcomes-based commissioning and capitation payments. This has been mirrored within the Life Science community through the AAR, which seeks to promote NHS collaboration and to develop a pathway to coordinate regulation, payment, evaluation and diffusion processes to get transformative treatments to patients quicker.[31] The setting up of a strategic commercial unit presents opportunities for 'novel risk-sharing agreements' between the NHS and the

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innovator. From both directions, therefore, there is significant interest in evolving a healthcare system that promotes greater innovation and focus on outcomes. The AAR also recommended that an Accelerated Access Partnership be established to help get new treatments to patients much quicker.[32] In its manifesto, the Conservative Party committed to 'implement the recommendations of the Accelerated Access Review to make sure that patients get new drugs and treatments faster while the NHS gets best value for money and remains at the forefront of innovation.'[33]

More specifically, a study by PwC for the AAR looked at 13 different reimbursement schemes. Stakeholders who responded to a survey as part of this analysis rated outcomes-based reimbursement as one of the most attractive schemes, and the idea achieved support across industry and Department of Health / NHS.[34] The report also assessed outcomes-based payments as being appropriate for most of its product archetypes and appropriate in a larger number of instances than other reimbursement mechanisms.[35]

At the same time, the concept of outcome-based commissioning has gained ground in public services. The idea has been adopted in various forms in private healthcare insurance markets as well as in the NHS.[36] However, while a number of one-off schemes have taken place, work is now needed to establish such an approach more securely and sustainably and make it a tool that commissioners can use appropriately. Despite a seminal study by the Office of Fair Trading in 2007 into value based pricing and a response by the Government in 2010, there has been no systematic development of the policy.[37] The ambition set out in 2010 to introduce value based pricing in 2014 was not crystalised. The claim that 'there must be a much closer link between the price the NHS pays and the value that a medicine delivers' has not been heeded.

In the analysis that follows, we draw on lessons from the past, from other countries and from other public services to sketch out how a system of outcomes-based reimbursement could function.

PART III: WHAT ARE THE MAIN CONSIDERATIONS IN DESIGNING A SCHEME?

This section assesses the main design considerations that policymakers would have to make in developing an outcome-based reimbursement model (OBRM) and how these could be managed. These include:

1. How to share outcome risk between the NHS and the manufacturer.
2. How the initial costs of drugs are shared.
3. What outcomes to pay for?
4. What drugs should be made available through the scheme?

1. Deciding how to share outcome risk between the government and the provider

Currently, outcome risk sits with the Government. Under OBRM, the risks could be shared between the NHS and the manufacturer. From a theoretical perspective, risks should only be passed to the manufacturer when the latter has some control over the outcomes.

In a scenario where outcome risk is passed to the manufacturer, the drug company would walk away with no revenue from the drug if the agreed outcomes were not achieved. From the state's perspective, this 'pure' version of payment by results would protect taxpayer resources. However, it may be beneficial for the purchaser and manufacturer to share the risks. If the drugs achieved better outcomes than expected, the manufacturer would benefit from the situation, which would help align incentives. Existing international and other public sector applications of the principle of payment for outcomes (or results) suggest that getting the correct distribution of risks is important.

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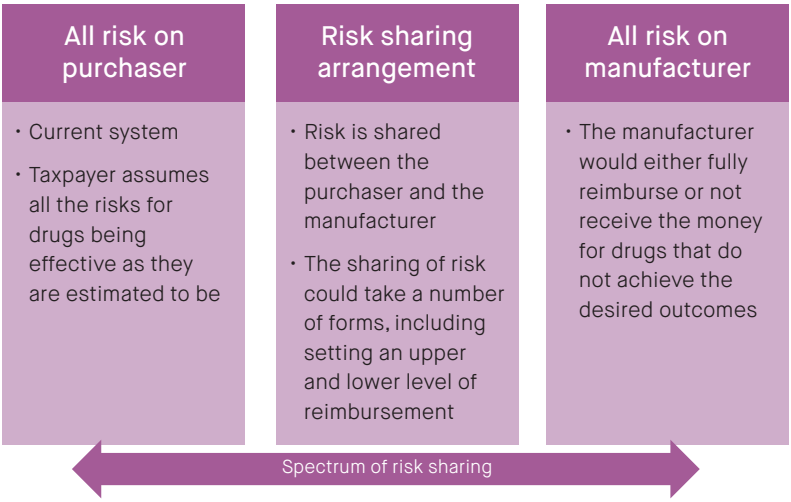
Figure 2: Risk sharing options



How existing schemes function

A number of past schemes in pharmaceuticals have required the manufacturer to make full refunds where outcomes are not achieved. However, it is also commonplace in public services and in pharmaceutical agreements for risks to be shared.[38] In drug schemes this has meant requiring price discounts and triggering free treatments in the event of the agreed outcomes not materialising. Risk sharing agreements have been used in drug reimbursement (see case study on Italy) and in payment by results schemes in the UK.[39]

- Offender rehabilitation: The contractors are paid a fee-for-service element for probation activities as well as a reward payment if an ex-offender desists from re-offending.
- Employment support: Work Programme providers were paid by results to get individuals into sustainable employment. The Department estimates that 80% of the payments to providers are on the basis of results over the course of the programme.[40]

- Rail franchising: The Department of Transport has often taken a share of the upside and the downside risk through a policy called 'cap and collar'. Here, where revenues exceed a given threshold then proceeds are shared between the provider and the government; the government also shares the losses if revenues fall below a given benchmark.[41] Such schemes may limit the incentive for rail firms to invest during the course of the contract.

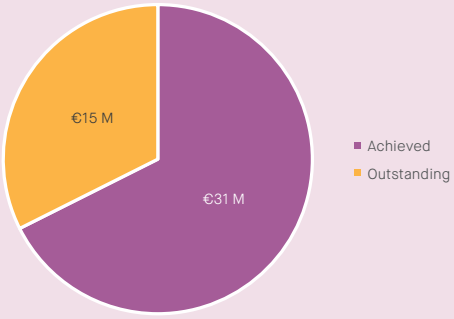
Box 2: Italian case study

Italy uses a number of managed entry agreements, some of which are similar in principle to our proposed OBRM. Three types of agreement can be stipulated, these are:

- Cost sharing (CS)
- Payments-by-results (PbR)
- Risk sharing (RS)

CS agreements involve price discounts and are not outcomes-based, whereas PbR and RS agreements involve outcome measures and involve reimbursement to the healthcare provider. When agreeing to PbR contracts the manufacturer is required to pay back the full amount for those patients who do not respond to treatment, known as 'non-responders'. This is an example of when the manufacturer takes all of the risk. Within the RS contracts, the Italian healthcare purchaser and the manufacturer share the burden of risk. These vary from PbR, as the manufacturer now has to pay back a proportion of the drug price for each non-responder. Non-responders have to be identified within a pre-set time for each drug; the median value is 8 weeks but can range from 4 to 12 weeks.[42] As of October 2012, there were 16 PbR contracts in place in comparison to two RS contracts, which shows the preference for the risk to be borne by the pharmaceutical sector. Research has suggested that the use of such arrangements is commonly associated with faster patient access to medicines in Italy.[43]

Figure 3: Value of Italian reimbursement



Source: L. Garattini et al., Italian risk-sharing agreements on drugs: are they worthwhile? (2014)

In September 2013, the AIFA (Italian Medicines Agency) published the revenues associated with the managed entry agreement. The total amount that theoretically should have been paid back to the commissioner was €46.3million. However, only two thirds of this sum was received and a third had not been received: 11% because of late requests for reimbursements (by hospitals) and 22% due to disputes with the pharmaceutical sector relating to the evidence base. Total refunds from manufacturers came in at around 5% of expenditure for the drugs involved.[44]

Due to the regional nature of the Italian healthcare system, there have been some difficulties in ensuring compliance with the reimbursement system. Prescribers have had to be reminded to ensure that all patients are correctly recorded on the national system and that payback procedures are applied correctly.[45] The case study emphasises the importance of clarity over outcome measures as well as ensuring that the right procedures are in place to boost compliance by administrators.[46]

Factors determining efficient distribution of risk

A number of factors are likely to determine the most efficient distribution of risk in an agreement. First, there are considerations to do with alignment of incentives and mutual effort towards improved patient outcomes. Under a risk-sharing scheme both the commissioner and the manufacturer have an incentive to recommend that a patient receives a drug where there is a reasonable likelihood that the patient's health will improve. Therefore, the interests of both the commissioner and the manufacturer are aligned in favour of appropriate patient selection. A similar lesson may apply where patients are in control of their own treatment. Here risk sharing may ensure that both the NHS and manufacturer is seeking to promote medication compliance among patients.

Second, the distribution of risks may affect the nature of the pharmaceutical market. In markets such as employment support and offender rehabilitation where payment by results have been introduced there have been concerns about the effect on smaller providers. Smaller providers (private or charitable) will find it harder to manage outcome risk because their balance sheets are less substantial. In addition, outcome risk may systematically be less appropriate because smaller firms may be dealing with smaller cohorts of patients where statistical significance may be an unassailable problem.[47]

Summary

Outcome risk can be shared between the purchaser and the manufacturer. However, the two parties may agree occasions where the risk should pass entirely over to the manufacturer. The distribution of risks between the two parties should be tailored to the specific intervention.

2. Deciding the payment schedule

A second important consideration is which party should cover the immediate costs of the interventions. In instances where outcome risks are passed to the manufacturer of the medicine, the state could decide to defer paying for the drugs until it has observed the outcomes. There are good reasons for considering such a step, although there are also associated risks that would have to be managed and that may make it inappropriate.

The options and the trade-offs

Outcome-based commissioning schemes typically deploy one of two methods of payment schedule. First, the NHS may cover the immediate costs of the interventions – in other words, the NHS finances the interventions. In the event that the manufacturer achieves the outcomes or better, then it retains the money it has already received with the potential for a performance benefit. In the event that the outcomes are not achieved, then the NHS claws back a portion of its initial expenditure as a penalty from the manufacturer. For instance, in some risk-sharing agreements in pharmaceuticals (e.g. Velcade for multiple myeloma and cetuximab for colorectal cancer) the manufacturer promises to refund the costs of treatments that do not work.

An alternative approach is for the manufacturer to cover the immediate costs and to finance the interventions. The NHS then pays the manufacturer when / if the agreed outcomes are observed. This is common practice in public service commissioning (offender rehabilitation, employment support), though not in pharmaceuticals. An important consideration is how the outcome is to be measured and the appropriate trigger for payment. For

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instance, living additional years is by definition a long-term measure and in such cases an alternative, surrogate trigger may be acceptable if this is closely correlated to the desired outcome (e.g. a clinical test that predicts a longer life).

In straightforward terms of economic efficiency, requiring the manufacturer to finance interventions that the state could otherwise finance implies a higher cost of borrowing. However, there may be countervailing benefits.

Table 1: Trade-offs – Advantages and disadvantages of the manufacturer covering the immediate costs of interventions

Potential advantages of the manufacturer covering immediate costs	Potential disadvantages of the manufacturer covering immediate costs
May alleviate funding pressure on short-term NHS budget and enable rationing to be less severe (for more detail see below)	The Government can borrow money at cheaper rates than the private sector.
Ensures that the purchaser is insured against the risks of a manufacturer going bust when it owes money to the state. This may be less of a concern for very large pharmaceutical firms, and more relevant for smaller firms and originators.	The full benefit of a drug may only be measurable over many years, thus putting a long-term financial obligation on the manufacturer. This may discourage firms and limit market participation. Smaller firms in particular may find it difficult to finance the interventions. Therefore, measures would need to be taken to guard against market concentration and preserve diversity and the role of smaller providers.
Eliminates risks associated with recouping payments from firms (for instance, having effective collection systems in place).[48]	May have an unintended consequence of the pharmaceutical industry being less able to invest in other new treatments. However, this would ultimately rely on the potential returns that investors foresee from investing in developing treatments for the UK market.

As can be seen from Table 1, there are significant disadvantages as well as advantages from requiring the manufacturer to cover its share of the costs of interventions. There may be circumstances when it would be optimal for the commissioner to defer payments but there are likely to be many considerations to take account of. Treatments often vary substantially and it is important that the system is flexible in order to treat some diseases and products differently. In its study for the AAR, the PwC found that along with therapeutic reference pricing, disease-specific P&R pathways and indication-based pricing, deferred payments was seen by stakeholders as among the least attractive new models.[49] From the public sector side, this may in part stem from the difficulties associated with the problems of fragmented budgets across different health and social care settings which may make it more difficult to realise the longer-term savings.[50] Single commissioning budgets at a local area could help address this dilemma in the medium term.

How changing the payment system could help manage the short-term NHS budget constraints

As it stands, the NHS pays for treatments when they are made available to patients. Altering this practice so that the manufacturer covers (some or all of) the immediate costs of treatment could have implications for the ability of the NHS to make treatments available to patients and could offer a route to achieve cost control as well as pursuing value.

All advanced economies face concerns about the growing costs of healthcare and, in particular, anxieties over the high costs of treating large patient groups with new expensive treatments. The response so far in the UK has been towards cost control rather than the pursuit of value.

Developments between 2015 and 2017 provide evidence of this effect. In 2015, NICE recommended ledipasvir-sofosbuvir for the treatment of chronic hepatitis C, a condition that affects 215,000 people within the UK. The new drug had the potential to cure patients. However, costs range from £26,000 to £78,000 depending upon the length of treatment needed. Due to budget constraints, NHS England decided to cap the number of patients who can access the drug each year to 10,000. The consequence is that many

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patients are denied access to the treatment for many years. Meanwhile, the NHS must spend money treating the patient in other ways in the intervening years, which will likely mean a higher cost overall. By 2020, the NHS plans to be treating 15,000 people with those with the highest unmet clinical need prioritised first.[51]

The case described above has also triggered wider policy measures to control costs. From 1 April 2017, NICE and NHSE have implemented a new budget impact test.[52] This will directly impact drugs where there is a sizable patient population and could add further delays to patient access. This threatens to sacrifice long-term value for short-term control.

In this context, altering the payment schedule could help overcome the issues associated with NHS annual budget constraints, particularly when a new innovative drug becomes available with potentially transformative properties for large numbers of patients.

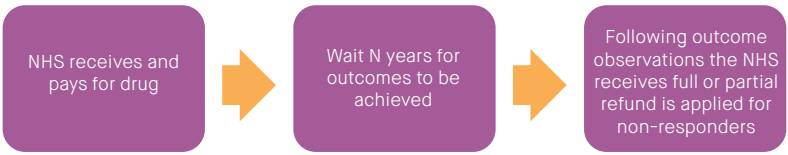
How a different payment schedule could work

If the NHS were to pay for outcomes only when they are observed, it could defer expenditure whilst making treatments immediately available. But, how could such a scheme work?

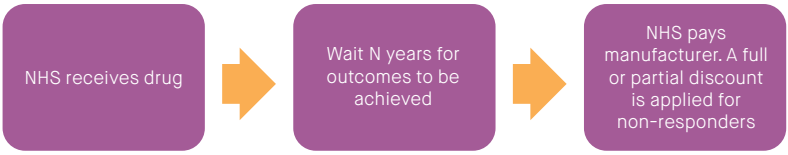
Potential impact of altering the payment schedules

Below we describe the potential effect of changing the payment schedule in instances where the observed outcomes cover more than one budget year.

Under the first payment example the NHS still pays upfront for the drugs it receives. Access to new treatments for patients is still achieved due to the introduction of outcome based reimbursement but the payment schedule is not influencing access.



Under the second payment schedule, the NHS no longer pays up front for the drugs it receives. This may be able to contribute to further improvements in patient access to new treatments. For instance, take a drug discovered for a cancer where there were 5,000 patients who had an unmet need, where the average costs of the treatment were £30,000 and where the outcome was five-year survival. If the manufacturer covered the initial costs of treating the population (£150m), the Government would be able to set aside £30m each year for five years to cover the final costs of reimbursement in Year 5 of £150m.



Box 4: Over what time periods would OBRM schemes run?

In a scenario where the receipt or reimbursement of payment will depend upon observed outcomes, the question of availability of outcome data and when it is available becomes paramount. The complexities associated with outcome measurements means that a one size fits all model will not work. For example:

- Nilotinib, which is used to treat chronic myeloid leukaemia, is also subject to a performance-linked reimbursement agreement in Italy, with the manufacturer agreeing to cover the costs of all treatments where the patient who does not reach an agreed haematological response after one month.[53]
- In Germany there is an outcomes-based pricing arrangement in place for zoledronic acid, which is used to treat osteoporosis. The manufacturer agreed to cover the cost of any patient who suffered a fracture within one year of being treated with zoledronic acid.

There are a number of medications where a one-year observation period would not be adequate in enabling outcomes to be measured. Some medications may require reimbursement contracts that relate to a number of courses of treatment rather than a specific time window. Other medications, particularly those that aim to increase life expectancy may require a longer period for outcomes to be observed. For instance, the UK performs poorly at five-year survival rates for cervical and colorectal cancer and this may be an important measure to attach payments to.[54] The multiple sclerosis scheme tracks patients over a ten-year period.[55]

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Summary

Who pays and when has important implications for the market and for the NHS budget. Asking the manufacturer to cover the immediate costs of the interventions would defer costs for the NHS and therefore enable it to make treatments available quicker, however it will not always be the optimal policy and there are significant challenges that would have to be overcome. Deciding who pays and when should be a flexible option in order to accurately react to difference in diseases and medications.

3. Deciding what outcomes we should pay for

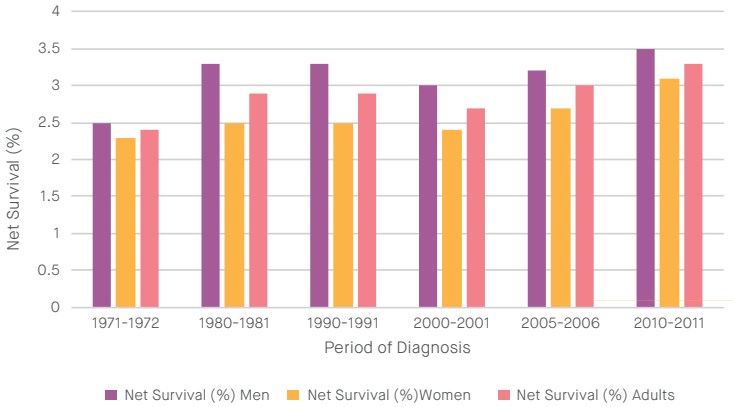
What to pay for

NICE is a respected body that implements a robust methodology. Since its inception, NICE has not only influenced what drugs are made available through the NHS but it has increasingly set global references points for pricing.[56] The QALY should provide the starting point for determining a unit of outcome to be achieved. However, there may be benefits to supplementing the QALY with broader measures of value, as described below.[57]

1. Putting additional value on 'unmet need'

Putting greater weight on 'unmet need' could send a signal to investors that they will be remunerated adequately if they bring innovative drugs to market whilst enabling more transformative drugs to be made available to patients. 'Unmet need' captures the extent to which a medication treats a disease population where there is currently no effective treatment. Levels of unmet need are high across a number of diseases. For instance, pancreatic cancer is the UK's fifth biggest cancer killer. Less than 5% of patients live for five years or more after diagnoses, a rate which has remained relatively unchanged for forty years, as shown by Figure 4.[58]

Figure 4: Pancreatic cancer 1971-2011; age standardised five-year net survival, England and Wales



Source: Cancer Research UK

2. Putting greater weight on quality of life and qualitative improvements

Charities representing patients with severe diseases frequently reinforce the importance of measuring the impact of a treatment on a person's quality of life and emotional well-being.[59] Equally, patient champions also emphasise the importance of putting value on the ability of patients with short life expectancy to spend time with their family even if this doesn't fit with the typical QALY definitions. This may include putting greater weight on more modest extensions to life expectancy where these have a significant impact on the quality of life of the patient or where life expectancy is otherwise very short.

3. Paying more for indirect benefits and positive externalities

Drug treatments can produce a number of positive benefits and externalities as well as delivering the direct benefits of improved patient health.

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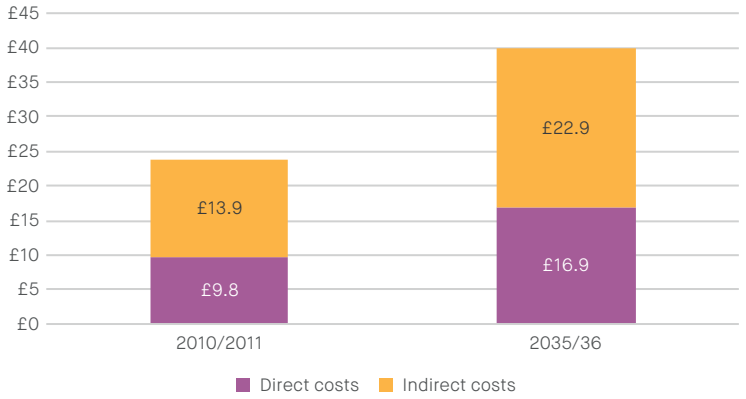
a) Medical costs

Interventions may contribute to cost savings associated with a reduction in the number of hospital stays or GP appointments. Some existing risk-sharing arrangements include a target set for a pharmaceutical firm by an insurer to reduce hospital admissions for heart failure.[60] In devising any such payment measures commissioners should be aware that improving someone's health for a year may not eliminate future costs of care but simply defer them. Past evidence has also suggested that reduced hospitalisation does not translate straightforwardly into cash savings unless a hospital can close a ward.[61]

b) Indirect costs:

Pricing mechanisms often fail to incorporate market externalities. While NICE evaluation includes an assessment of the patient's expected productivity, the indirect effects on other individuals is usually not incorporated.[62] An example of such externalities is the impact that drug provision has on those providing unpaid caring services. Academics at Sheffield University have estimated that in 2015 the value of unpaid care equated to £132 billion per year.[63] An improvement in patient health may release some of this value back into the formal economy if the carer is of working age. There are a number of diseases where indirect costs are significantly higher than the costs associated directly with the treatment and management of the disease. This is the case with diabetes. In 2010/11, direct costs were estimated to be £9.8bn, which is significantly lower than the estimated indirect costs, which stood at £13.9bn. In these estimations, indirect costs were calculated from data on mortality rates, sickness, presenteeism and informal care. Figure 5 shows the estimation of costs in 2010/11 and forecasts ahead to 2035/36. The proportion of total costs attributed to indirect costs is approximately equal over time.

Figure 5: Direct and indirect costs of diabetes in the UK (£bn)



Source: http://www.talkondiabetes.org/docsTOD/j.1464-5491_.2012_.03698_.x_.pdf

Including indirect costs could have a number of positive effects particularly for the economy and for individuals who are supporting patients during their treatment. This approach should incentivise manufacturers to produce medications that are effective at all stages of disease and that can positively affect the lives of those caring for patients. The challenge is deciding which indirect costs to include.

c) Weighing up broader outcomes

Countries including Sweden and Norway incorporate societal perspectives into their costings process: they are willing to pay more for products with larger social impacts. However, there is little evidence thus far to indicate that this has made a significant difference to the prices paid.[64] Research suggests that the productivity gained must exceed patient consumption if this is to have a positive net effect on society.[65] It is difficult to value these costs in monetary terms. In Sweden the human capital approach is used, which in theory values all uses of time: utility associated with leisure, education and retirement should be included. An alternative option is the friction cost method; this only considers temporary costs that are due to reduced working capacity.[66]

It remains unclear what role the economic and fiscal evaluations should play in the reformed process. Whilst these measures capture a number of costs and benefits that are not normally reviewed, difficulty can arise when trying to assign monetary values to these outcomes, adding complexity as well as opening moral issues around whether society should prioritise patients with certain characteristics over others. In re-assessing what we pay for we do not advise that matters of social justice should give way in favour of a purely economic equation.

Summary

NICE's QALY methodology provides a good basis for what commissioners should be ready to pay for. However, there may be scope to build on QALY to capture wider value that derives from drugs, including savings to the NHS, productivity improvements and wider impact on society.

What to measure

As well as deciding which outcomes to pay for, commissioners must identify measures that can inform them whether or not these outcomes have been achieved. Here, they may face a trade-off between what they would ideally measure versus what is practicable to measure. A number of past schemes have engineered measures that are strong in theory but vulnerable in practice. For instance, the outcome measure developed for the Velcade scheme for multiple myeloma centred round a refund offered by the manufacturer if levels of abnormal M-protein found in patients' blood failed to decrease by a pre-agreed percentage. However, there was subsequently significant disagreement over the optimal level of thresholds response.[67] It will be important for stakeholders to input into the development of appropriate outcomes measures in the context of clinical data, clinical practice and the practicalities of collecting reliable data.

In a perfect world, the NHS would acquire information that is real-time, relevant to the actual outcomes (rather than proxies) and that encompasses measures across all outcomes in which society is interested. As Michael Porter has argued, outcomes are condition-specific and multi-dimensional.[68] However, data may be costly to gather and administer, insufficiently timely

and in some cases impossible to acquire. Moreover, it may require a complex algorithm which would introduce additional complexity into the process.

Research by National Voices and the Health Foundation has argued in favour of the NHS co-producing measures with patients as a necessary step towards person-centred care.[69] In particular there may be a tendency to track what we know we can already or easily measure rather than what matters to patient outcomes (what has been termed 'outcome first/patient second').

Figure 6: Four dimensions of outcome measures[70]

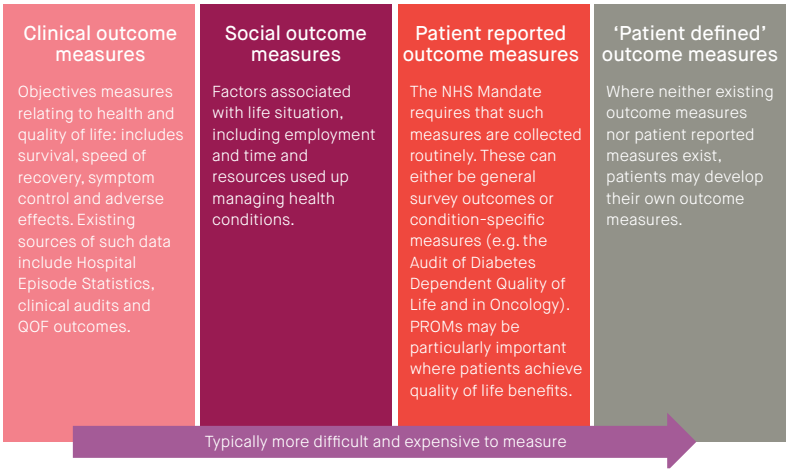


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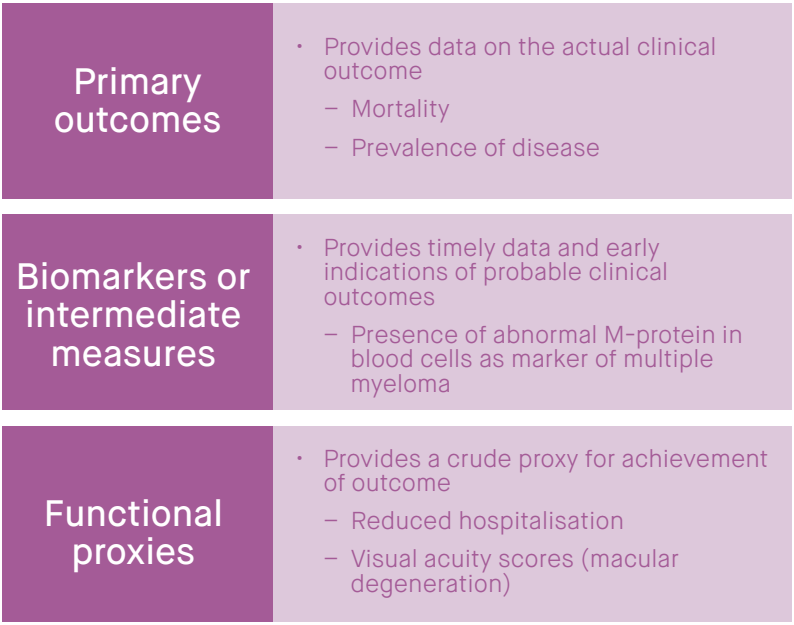
Figure 7: Hierarchy of outcome measurements



Summary

Ultimately, the balance must often be struck between simplicity, transparency and timeliness on one hand versus data quality and cost on the other. Commissioners would be wise to err on the side of simplicity.

4. Deciding what drugs should be covered through the scheme

It is easy to see outcomes-based schemes as a 'silver bullet'. However, as past analyses have concluded, and as indicated above, it can be time-consuming to negotiate, expensive to administer and difficult to assess.[71]

OBRM is a comparatively expensive and resource-intensive way of procuring goods and services because it requires significant information and measurement as well as investment in commissioning design and time commitment from the purchaser and the provider. It would be an inefficient commissioning method for goods and services where there is a high level of

certainty about what outcomes would be delivered by a given intervention. You would not procure tarmac for a new road through an outcomes-based contract.

If all drugs should not proceed through the OBRM, what qualifying conditions should drugs fulfil to participate?

- **Condition 1: Where uncertainty is high.** OBRM would be more suitable for new drugs where the impact is highly uncertain. This would include new drugs that are untested in real situations and therefore where the true impact is unknown. In such instances, the NHS would no longer be liable to pay for medications that proved to be ineffective, and therefore it may be ready to make drugs available to patients quicker.
- **Condition 2: Where the new drug addresses 'unmet need'.** Unmet need is an area that is currently not taken into account during the appraisal of drugs for routine commissioning. Making breakthrough drugs available to patient populations is a high priority and therefore they could automatically qualify to progress through the OBRM. In many cases, we may expect there to be significant crossover between the drugs that meet condition 1 and 2 (i.e. highly innovative drugs are likely to be associated with uncertainty and have the potential to address issues of unmet need).
- **Condition 3: Where the aggregate costs of administering the reimbursement model are proportionate.** Given the costs of administering OBRM, it should only be pursued where the aggregate costs of the medicine for the patient population is significant.
- **Condition 4: Where there is a reasonable prospect of outcomes being defined and measured.** For instance, critics have argued that a risk-sharing agreement for MS has suffered due to the difficulty measuring disability progression, thus resulting in inconclusive price reviews, whilst the ten-year period was considered too long by some critics.[72] However, more recent analyses have pointed to successes including predicting a positive long-term effect of the disease-modifying therapies among MS patients consistent with UK cost-effectiveness requirements.[73]

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The size of the patient group is also likely to affect the prospect of calculating a statistically significant result. Where patient groups are very small, there may be problems knowing whether or not the treatments have had an effect.

Summary

Establishing clear conditions for drugs to be considered through outcomes-based commissioning will be an important step towards provider certainty to investors and sustainability in the system.

PART IV: WHAT PRINCIPLES SHOULD GUIDE THE PROCESS?

This section describes the working principles of an outcomes-based scheme. As far as possible, in introducing greater flexibility we should build on the strengths of the UK system. We are proposing an alternative formal route through which new drugs can pass for approval. Decision points must be as transparent and clear as possible to send clear signals to investors. Layering in such a scheme would have the benefit of minimising disruption to current processes. As discussed in this report, and elsewhere, existing experience with outcomes-based approaches suggests that success is heavily reliant on good design scheme, including how outcomes should be measured and assessed, how evidence will be gathered and how results will be reported and governed.[74]

Table 2: Principles that could underlie a scheme

Step	Description
Qualifying criterion: Does the drug address an 'Unmet' need?	Based on evidence provided by the manufacturer, any clinical trials and other existing data, NICE and the NHS are likely to be more interested in drugs that can meet 'unmet need'.
Alternative qualifying criterion: Does the drug provide wider value that the QALY does not always capture?	The manufacturer has relevant evidence that the drug can achieve high levels of wider benefits not always captured in QALY.
Practical checks: Is outcomes-based scheme practicable?	The purchaser (for example NHS, clinicians, commissioners, NICE) and the manufacturer agree on the practicalities of an outcomes-based approach, for example: Which outcomes are relevant. • Whether relevant outcomes can be easily observed and measured, including whether the data is available and from what sources. Absolute clarity is needed on what will be measured and when, so as to reduce the likelihood of disputes.[75]

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Table 2 *Continued*

Step	Description
	• Whether the patient group is large enough to provide results that are definitive and statistically robust, providing the commissioner with requisite evidence on which to make payments.[76] • Whether likely expenditure on the drug is large enough to make administrative costs of the process proportionate. • Where is the scheme implemented – i.e. locally, regionally or nationally.
Decision on most appropriate route: Should the outcome agreement be part of the Health Technology Appraisal assessment?	If a national scheme is being considered, then appropriate agreements should be in place that allow the appraising body (e.g. NICE) to consider the outcomes-based scheme as part of the overall HTA evaluation if appropriate. The decision on whether to implement an outcomes based scheme could occur at a number of points during the appraisal process, as is the case when deciding on whether drugs should be available through the CDF.[77]
Agreement terms: how should risks be shared and what are the payment terms?	The NHS sets the terms on which the drugs can be procured, including: • The outcomes that need to be met (e.g. patient health and well-being, hospital admissions), when they need to be met and how they will be observed. • The commercial agreement covering when the outcome is achieved/not achieved. • How risks will be shared. • The flow of money. • Any other commercial considerations such as confidentiality.

Table 2 *Continued*

Step	Description
Drug is made available to patients	• Assuming an effective risk-sharing agreement is put in place, it should be in the interests of both the commissioner and the drug company to ensure that the drug is used appropriately on the right target patients. • The firm is likely to be of assistance in recommending how products fit into care pathways and patient selection. • At the same time, data collection processes are established and agreed to ensure that outcomes can be tracked.
Relevant outcomes measured	At the end of the outcome period, the outcomes are measured and recorded. It is important to consider who has responsibility for collecting the data and who subsequently owns the data.
Reimbursement contingent on the agreed outcomes being met/not met	The reimbursement is completed on the basis of the agreement. Payment will be made based on health value multiplied by number of patients or on the basis of a rebate as previously agreed. Past evidence suggests that it is important to institute mechanisms to ensure that reimbursement functions effectively especially if this relies on information gathered by third parties.[78] The process could be led by NHS England's Strategic Commercial Unit. Schemes will need independent oversight and governance.

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PART V: HOW COULD WE MANAGE SPECIFIC DESIGN CHALLENGES?

This section describes additional design considerations and how the wider healthcare system needs to change.

Ensuring that the necessary outcome data is available

Data availability

Under an outcome-based model, the NHS needs access to excellent data on health outcomes. There is anecdotal evidence that data shortages and lack of confidence about data availability has undermined the UK's readiness and ability to engage on innovative reimbursement structures through the Patient Access Scheme.

The UK has strengthened its healthcare information architecture and accountability considerably. Examples of current measurement practices include: the NHS Outcomes Framework which covers five dimensions of care outcomes; and data developed through the Health and Social Care Information Centre and by inspectors such as the Care Quality Commission. NHS England is developing Clinical Services Quality Measures across a range of priority areas including stroke, cancers, cardiac and mental health (dementia and psychosis).[79] These are composite measures based on multiple data sources aimed to provide information to patients, commissioners and the NHS.

However, despite these improvements, commissioners moving to outcome-based commissioning have encountered challenges relating to the availability of appropriate data. One study concluded that for a typical patient segment, data exists which allows 50-60% of outcomes to be measured.[80] Many schemes have had to establish new data collection systems to facilitate new commissioning practices.[81] Early schemes designed to introduce outcomes-based reimbursement required significant upfront work to improve outcome measures.[82]

Filling data gaps and introducing robust mechanisms are significant undertakings. Moreover, data collection necessarily imposes a burden on clinicians, commissioners and indeed patients. However, this is a journey that the NHS is embarking on already and needs to be pursued as part of the wider reform agenda. Innovative initiatives are underway in a number of local health economies. Through its Centre for Cancer Outcomes, the Cancer Vanguard at UCLH project is aiming to identify relevant outcomes in each disease area using a multi-disciplinary team scorecard and assessing data quality and completeness.[83] As past work by the SMF and others has argued, better health outcome data can also drive stronger accountability and benchmarking as well as enabling innovative commissioning and early identification of the causes of health care variation.[84]

There is considerable work underway at a local, national and international level.

- **STPs and digital ambition:** The STPs contain significant data ambitions. The Great North Care Record and the Hampshire Health Record are examples of advanced practice in this area. In Hampshire, the health record includes 87 million documents and has approximately 8,500 unique users. The data covers areas such as GP diagnoses, blood and radiology results, current medication and some social care information.[85] The North Central London has published a Local Digital Roadmap.[86] This could include utilising data from wearable devices. The area is establishing a 'NCL Population Heath Management Model' which will 'monitor health outcomes and treatment effectiveness to enable value based commissioning' by 2020.
- **Use of and development of outcome measures for outcomes-based commissioning.** Bedfordshire developed an outcome-based capitated payment contract for musculoskeletal conditions, with a provider paid by results. The CCG adopted the ICHOM Standard Set for low back pain which had been developed by physicians, care specialists and patients.[87]
- Development of standard outcomes measures. A recent initiative has produced a standardised set of outcome measures of colorectal cancer. Colorectal cancer is the third leading cancer in men and the

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second in women globally.[88] ICHOM has created a set of standard outcome measures that are applied across a range of diseases. In Colorectal cancer, the outcomes include: disutility of care, degree of health, survival and disease control and quality of death.[89] In oncology, the Systemic Anti-Cancer Therapy dataset collects standard clinical management information on patients undergoing Chemotherapy funded by the NHS in England.[90] This contains information from hospital electronic prescribing systems and is linked to the Cancer Outcomes and Services Database, the Radiotherapy Data Set and three diagnostic databases.[91]

There remain many areas for further work. First, efforts need to continue to develop standardised methods for capturing patient insight and feedback as well as enabling patients themselves to help develop the outcome measures. Second, while there is significant work going on at a local level, NHS England will need to play a strong coordinating role so as to ensure that data gaps are filled, to reduce variation and to ensure common standards and measures where possible. Ultimately, the local NHS will need to demonstrate that it has the right data collection tools and policies to enable it to track relevant outcomes before it is able to purchase these treatments in its area. Common processes for responding to data collection gaps and variation could help.

Third, NHS England and NICE should consider how they can work with the pharmaceutical industry and patient charities to improve data collection and completion. This should start with a working group being established incorporating drug manufacturers, providers, commissioners, clinicians, patients and officials to oversee an audit of data gaps and set benchmarks for local commissioners and providers.[92] A desirable outcome would be a data collection framework which could guide what data is collected, when and how. Further steps could also help including cross-sector efforts to improve NHS IT infrastructure so that reliable outcomes data can be recorded across the country. As the ongoing work by the ABPI on Real World Evidence highlights, further collaborative work is needed between the NHS and industry to build an infrastructure to support linked, multi-source datasets. Meanwhile, patients themselves must be engaged in deciding how their data is used.[93]

Information architecture

Decision-making credibility is extremely important, including achieving and retaining the trust of patients, clinicians, politicians, commissioners and investors. Decisions on whether outcomes have been achieved must be made independent of government and commercial interests – so as to reduce the risk of fraud and limit the influence of external factors on decisions.[94] The SMF has previously advocated establishing an Office for Patient Outcomes. Such a body could provide oversight as well as arbitration where there are disputes over the interpretation of results.

Good data on outcomes is crucial to effective reimbursement as well as to the future of outcomes-based commissioning more widely in the NHS. The Government should institute an audit of NHS data gaps and ensure that any data gaps are identified and closed. This should be overseen by a cross-industry working group.

Assessing attribution and ensuring that providers are rewarded appropriately

Attribution risk

In designing new reimbursement methods, consideration should be given to attribution – in other words what factors have contributed to the observed outcome. Manufacturers want to be confident that the effect of their interventions is rewarded appropriately.

Economic theory would suggest that the optimal incentive structure would be for all factors that contribute to an outcome to be in the control of the agency that is being incentivised.[95] As Porter has argued, 'the proper unit for measuring value should encompass all services or activities that jointly determine success in meeting a set of patient needs.' This is the logic behind policymakers seeking to integrate responsibilities for health and social care in one place so that commissioners can choose the right mix of interventions to improve outcomes.[96]

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Health outcomes may be affected by lifestyle factors and compliance with prescriptions. Ensuring that good data is captured on whether patients are taking their medicines as prescribed will be important. There may also be concerns over systematic bias. Although our research has not unearthed instances, it would be possible to imagine a scenario in which there was systematic bias that worked in favour or against the performance of a drug. This could occur if forward-looking and innovative clinicians were more likely to prescribe better drugs than their peers. If these same clinicians also provided a higher quality of care in other ways to their patients then the observed outcome may be a consequence of who is prescribing a drug as well as what is being prescribed. We are not aware of current value-based pricing schemes seeking to control for all such factors.

The NHS may also want to consider external factors that may affect the likelihood of the outcomes being achieved. For instance, the wider state of the economy is likely to affect whether employment outcomes are achieved.

What happens if a patient stops taking a drug or takes an additional drug?

A particular attribution dilemma transpires when a patient takes multiple drugs or where a patient switches from Drug A to Drug B.[97] In such cases, the NHS and the manufacturer may be unable to disaggregate the effects of individual drugs on the outcomes. In part this can be controlled, by defining specific payment terms in these cases in the contract. However, in a mature scheme it may be possible for the firm that has developed Drug B to buy out the outcomes for the firm that developed Drug A.

> In introducing such a scheme, the NHS should evaluate whether there are significant differences in prescribing practices and whether there is correlation with other factors that may affect health outcomes.

Ensuring compatibility with devolved commissioning and local discretion

Looking further to the future, policymakers should also be open to more localised agreements to address the needs of a specific locality. Accountability for health and social care outcomes is increasingly being

devolved to local commissioners. For instance, since 2016 the Greater Manchester Health and Social Care Partnership has had control over a devolved budget for health and social care. Their plans include 'moving to outcome-based, multi-year capitation models that support implementation of new models of provider collaboration and innovation.'[98]

In a scenario where the local NHS has adopted outcome-based commissioning contracts and capitation payments, they may also want to play a fuller role in determining the price they are willing to pay for the outcome.[99] One of the earliest outcomes-based commissioning contracts in the UK was pursued by North Staffordshire Health Authority in 1999. This year, it has been reported that Manchester health leaders are considering paying pharmaceutical firms for medicines based on how well they work, using patient data to test real-world outcomes.[100]

Different local economies may have different priorities, different care pathways and different population needs. Local commissioners may want greater discretion over the outcomes that they pay for and amount they pay. For instance, one area may wish to prioritise reduction in hospital admissions whilst another may have particular policies focused on carers re-entering the labour market. It is therefore important that any policies around outcome schemes are future proof, with flexibility to allow for a national approach as well as regional and local agreements and implementation.

The personalised medicine agenda may drive this approach – manufacturers may be looking for healthcare partners with whom they can work to develop drugs around the needs and characteristics of much smaller patient groups. Many of these partners will be local collaborations (e.g. research intensive universities and hospitals).

Helping guide and develop the market

Part III demonstrated some of the trade-offs implicit in introducing outcomes-based commissioning in terms of whether such schemes could inherently favour larger firms whilst disadvantaging smaller firms. We might expect the market to achieve a new equilibrium as the reforms mature – for instance, smaller firms with good prospects that could not bring such

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schemes to market themselves may become likely contenders for buy-outs at higher prices.

The Government could consider making specific allowances for smaller firms. This is likely to be particularly important in terms of expectations on the manufacturer to finance the interventions – this could have significant implications for the cash flow of a small firm. The efficient distribution of risk in the case of a small charity may also be different. As a review for the AAR noted, innovative smaller manufacturers may not be well placed to provide free products for extended periods.[101]

One potential downside is that the system becomes more not less complex. This would give an advantage to larger firms that can invest larger sums in navigating the processes and regulatory complexities.

Any model should be designed with simplicity in mind – to send clear signals to investors and to retain diversity in the market where small providers can participate fully.

The Government should also consider making special allowances for smaller providers to allow them to participate. These should be made explicit so that firms come forward and propose new treatments. Special treatment could include: putting a lower level of outcome risk on smaller providers; and not expecting that smaller providers should cover the upfront costs of treatments.

Piloting the scheme and controlling costs

The NHS may wish to set parameters for the scheme to provide control on costs. This could be achieved via three possible routes:

1. The Government could pilot OBRM for a set period of time before reviewing the efficiency of the scheme as well as the costs of the overall medicine budget. The pilot would have to run for a sufficiently long period to allow data on outcomes to be measured and for the reimbursement cycle to take place. With this in mind we would propose

that any pilot should run for a minimum of five years. It would also be beneficial for any pilot to have coverage across different diseases and open to a variety of commissioning practices.

2. An annual budget limit on the value of outcomes purchased through the scheme could be set by central Government or, as accountability is devolved, by local commissioners. Such a policy would be vulnerable as the CDF was to pressure for expansion to enable access to additional drugs.

3. The generosity of the reimbursement mechanism could be adjusted up or down. As Part II described, the NHS would decide which outcomes to remunerate. Beyond the QALY, there may be a case to pay a premium for treatments that address 'unmet' need, that contribute to cost savings elsewhere in the system or that provide wider, less direct benefits to patients or carers. One approach may be to introduce the scheme at a lower level of generosity and increase it over time.

In considering how OBRM should be introduced, consideration should also be given to how stability, transparency and sustainability can be embedded.

How OBRM would sit alongside other initiatives

The policy described above would support the conditional approval and evidence generation features of the Cancer Drugs Fund, and would expand the current Patient Access Scheme (PAS) beyond simple discounts. The Early Access to Medicines would be outside of this scope as the product is provided free of charge pre-licence.

By putting these practical measures in place and adopting an outcomes-based approach, we can improve access and outcomes for patients, and maximise the NHS budget by applying the principle of only paying for what works.

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Outcomes-based reimbursement of medicines

This report describes the benefits of introducing an outcomes-based model for drug purchasing as a mechanism to address the affordability challenge, to reduce risks within the NHS and to improve outcomes for patients. This would apply the principle of the NHS only paying for what works.

The report describes how such a scheme could be introduced and the design challenges to be overcome.

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