

# Making Outcome-Based Payment a Reality in the NHS: Executive Summary

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in Greater Manchester



Together we will beat cancer

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# Reference

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Cancer Research UK and Greater Manchester Health and Social Care Partnership commissioned the Office of Health Economics, RAND Europe and Kings College London to carry out the research underpinning this report.

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# Cancer Research UK

Cancer Research UK is the world's largest independent cancer charity dedicated to saving lives through research. We support research into all aspects of cancer through the work of over 4,000 scientists, doctors and nurses. In 2017/2018, we spent £423 million on research institutes, hospitals and universities across the UK. We receive no funding from Government for our research.

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CANCER  
RESEARCH  
UK

CANCER POLICY  
RESEARCH CENTRE

Cancer Research UK is a registered charity in England and Wales (1089464), Scotland (SC041666) and the Isle of Man (1103)

<http://www.cancerresearchuk.org/>

## Full Report

The complete version of this report, including details of the underpinning research, is available for download from **[cruk.org/policy-drugs](http://cruk.org/policy-drugs)**

# Foreword

Cancer Research UK's ambition is for three in four patients to survive their cancer for 10 years or more by 2034. This will require diagnosing more cancers earlier, when treatment is more likely to be successful. But even for cancers diagnosed at the same disease stage, UK cancer patients' survival still lags behind comparable countries internationally. This suggests more could also be done to ensure every patient is receiving the best evidence-based treatment.

Cancer medicines are a crucial part of many patients' treatment and care, and access to these drugs is a hugely emotive issue for people affected by cancer and the wider public. Recent years have seen real improvements in UK patients' access to newly-launched cancer drugs. As this report sets out, exploring more flexible ways to pay for some cancer medicines, such as outcome-based payment (OBP), holds exciting potential to keep improving access to drugs by linking a drug's price to the outcomes it delivers for patients in the NHS.

Both the October 2016 Accelerated Access Review and the August 2017 Life Sciences Industrial Strategy called on Government and the NHS to implement flexible pricing models to support quicker adoption of innovations. And the new Voluntary Scheme for Branded Medicines Pricing and Access – an agreement between the Government and pharmaceutical companies which came into effect at the start of 2019 – committed to increasing commercial flexibilities for companies whose products offer significant value for the NHS.

The increasing number of cancer patients, and the intensity of care they receive, means resources must be spent on interventions that genuinely improve patient outcomes and experience. And our understanding of cancer as a disease is constantly evolving, leading to newer, more personalised treatments such as precision medicines and immunotherapies, but also adding complexity and cost. We know that cancers change over time and can become less responsive to individual medicines, and for many cancer types there are multiple drugs now available at different points in the patient pathway.

We believe OBP provides an important extra option which can be used when the NHS and a company cannot quickly agree a single, fixed price for a new cancer drug, and prolonged negotiations risk delaying or even limiting patient access. There are several trends which will make an OBP approach valuable and, importantly, realistic in the near future:

1. An increasing recognition that evidence of a drug's effectiveness from clinical trials – while essential to prove a drug's safety and efficacy – may not always reflect a medicine's benefits to patients in a routine clinical setting. This may lead to a greater emphasis on using real-world data of patients' treatment outcomes to agree a price that better reflects the drug's true benefit to NHS patients.
2. Many drugs are now being considered for use in the NHS with less mature clinical trials data on their effectiveness than in the past. Innovations should reach patients quickly, but this increases uncertainty about the drug's appropriate price. Complementing clinical trials data with real-world evidence could help maintain quicker patient access while still capturing the drug's long-term benefits, to help judge its value to patients. The data environment in cancer is more advanced than in many other disease areas, making it easier to achieve this.

3. Many of the latest cancer drugs are more complex and expensive than past medicines. This creates greater financial risk to both the NHS and manufacturers from agreeing a price which does not reflect the drug's true value, making negotiations to agree a single fixed price more difficult.

Implementing OBP requires understanding the treatment outcomes that matter most to patients, including factors beyond purely physical health outcomes. This research captures a range of these factors in the outcomes “flower” developed in our research, and which is shown throughout this report. Although what matters to patients will differ across a range of characteristics, people affected by cancer we surveyed identified a common core of priority outcomes to form the basis for an outcome-based programme.

The gain for patients from this new way of paying for cancer drugs is potentially twofold: faster access to innovative drugs where current pricing mechanisms are insufficient; and a greater focus on building NHS structures and services around accurate and explicit measures of the value that they receive from their treatment. Both of these factors should ultimately help to drive improvements in patient outcomes.

In Greater Manchester specifically, cancer incidence rates have historically been above the national average. But the devolution agreement, signed in 2014, provides an opportunity for innovations to be trialled locally, and for the region's health and social care institutions to work together more closely. All of this makes Greater Manchester a fantastic test bed for the kind of emerging, challenging thinking which will be required to design an OBP system for cancer medicines, which could then be feasibly tested in practice.

OBP schemes have existed in the NHS previously, but they have not been used systematically, in part due to a lack of consensus between all the relevant parties. We're pleased to have brought together a range of stakeholders – including government, NHS England, arm's-length bodies, the pharmaceutical industry, and crucially people affected by cancer – to develop a shared vision on this topic for the first time.

This report is the culmination of the first phase of our research in this area. We look forward to continuing to work with our partners to identify and overcome the barriers to implementing OBP within the NHS in England in the next stage of our research.

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# Executive Summary

## Cancer Drugs Access and Pricing in the UK

More than 360,000 people are diagnosed with cancer in the UK every year, and it's estimated that the UK spends around £2 billion each year on cancer medicines. In England, around 28% of all patients receive cancer drugs as part of their primary treatment, and this proportion is significantly higher for those diagnosed with advanced disease. In order to ensure the best quality care for these patients, it is vital they are able to access the most innovative and effective medicines for their condition.

After their safety and efficacy are proven in clinical trials, all new cancer drugs are reviewed through a process called health technology assessment (HTA), led by the National Institute for Health and Care Excellence (NICE) or the Scottish Medicines Consortium (SMC), to judge if the drug is value for money. If the medicine is not cost effective at the price initially proposed by the manufacturer, they can negotiate a different pricing arrangement. Many negotiations currently conclude with a simple percentage discount on the medicine's price.

Moves to bring new medicines to patients as early as possible are positive. However, this can contribute to uncertainty about the benefits the medicine offers to patients compared to existing treatment options, as the full evidence is still emerging. A drug's benefit may also differ in real-world healthcare practice to what was found in clinical trials. This therefore introduces uncertainty about what the NHS should pay.

Together with the increasing cost and complexity of new medicines, this may make it harder for the NHS and manufacturers to agree a single price for a medicine, potentially resulting in delays in patient access. More flexible ways for the NHS to pay for medicines could, in part, provide a solution.

Figure E1: Rationale for flexible pricing



Cancer Research UK and Greater Manchester Health and Social Care Partnership (GMHSCP) commissioned the Office of Health Economics and RAND Europe, in collaboration with Professor Richard Sullivan of King's College London, to explore the feasibility of introducing one type of flexible payment mechanism – outcome-based payment (OBP) – for cancer medicines into the NHS in England. This model links the price the NHS pays for a medicine to the outcomes it achieves in practice for NHS patients.

OBP could help to accelerate patient access to some new medicines and ensure close monitoring of real-world patient benefit. It can also promote value for money in NHS spending and support innovation emerging from manufacturers. This is especially valuable against the backdrop of rising overall NHS spending on medicines, and of the uncertainty created by the UK's imminent withdrawal from the European Union.

The research focused on establishing the treatment outcomes people affected by cancer consider most important, to inform an OBP approach. It included literature reviews, interviews with stakeholders, focus groups and a survey of cancer patients and carers.

Based on our findings and analysis we make several recommendations for taking forward OBP for cancer medicines both within Greater Manchester (with its devolved responsibility for NHS and social care) and at a national level. We have focused on specific arrangements in the NHS in England, including the national cancer data infrastructure, which represents a key foundation for any OBP scheme. However, our findings and conclusions remain relevant to decision-makers in the other UK nations and health care systems internationally.

## Defining Outcome-Based Payment

Outcome-based payment (OBP) schemes are commercial arrangements where a medicine's price is linked to the outcomes achieved for patients receiving the medicine in real-world clinical practice. Medicines that perform as expected and deliver pre-agreed outcomes are reimbursed at the pre-agreed price, while medicines that do not deliver on these outcomes are reimbursed at a lower price or not at all.

This definition encompasses a range of different possible models identified in our literature review, listed in Table E1, which vary in characteristics including how financial risk is shared between the company and the payer (i.e. the NHS in the UK context), and whether the link is dependent on population-level or individual patient outcomes.

**Table E1: OBP scheme categories and definitions**

Scheme category	Definition
Cost sharing arrangements	Price reduction for initial treatment cycles until it is clear whether a patient is responding to the medicine.
Payment-by-results	Manufacturers reimburse the payer in full in instances where the patient does not respond to the treatment.
Risk sharing	Manufacturers reimburse a proportion of the cost of the medicine for non-responders.
Outcomes guarantees / pay-for-performance	Manufacturer provides rebates, refunds or price adjustments if the medicine fails to meet pre-agreed outcome targets at the individual patient level.
Coverage with evidence development	Access to a drug is initially provided on the condition that further population-level evidence is gathered. Based on this further evidence the payer then makes a decision whether to continue funding the treatment or not.
Conditional treatment continuation	Payment for the continued use of a given drug is based on intermediate endpoints at the individual patient level.

OBP schemes are already in use in the UK, for cancer and non-cancer medicines. For example, in November 2017, NHS England announced it had agreed “pay by cure” deals for medicines to treat Hepatitis C and Multiple Sclerosis, which it badged as the latest in “a series of outcome-based payment arrangements”. There are also numerous examples of OBP schemes being used globally in countries including Australia, Italy and the Netherlands. These examples suggest that wider implementation of OBP for cancer medicines in the NHS is possible.

It is worth noting the reformed Cancer Drugs Fund (CDF), allowing NICE to conditionally approve cancer medicines and collect real-world evidence of their benefits (for use alongside clinical trials data in a later HTA reassessment), is effectively a type of OBP scheme, a form of “coverage with evidence development”. Over 7,500 patients received “managed access” drugs in this way between July 2016 and September 2018, demonstrating the value of this flexibility.

## Scope of Outcome-Based Payment Use

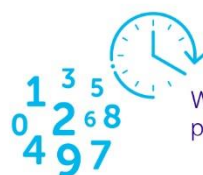
Our research also identified challenges to successfully designing and implementing OBP schemes. These included the timeliness and quality of the real-world data collected; concerns around administrative complexity; and ensuring there is consensus from both payers and manufacturers on the outcomes which will be used to determine price.

However, there was consensus among stakeholders we interviewed that these challenges could be overcome if all parties have the will to do so, and there is a clear benefit to patients, the NHS and industry. This suggests that while OBP may be unnecessarily complex for many medicines, it can play a role in facilitating patient access where a simple fixed price cannot be negotiated in good time – for example where there is uncertainty about a drug’s effectiveness based on clinical trials data, but the drug is felt to offer a reasonable prospect of significant clinical benefit in practice in the NHS.

Figure E2: Characteristics of medicines suitable for OBP



When the NHS and the manufacturer cannot agree a price in good time



When there is early but promising clinical trials data



When there is a small to mid-sized patient population



With a cancer type where improvements in outcomes can be captured in the short-run

In the interests of transparency, and to help ensure and monitor good practice in the design of OBP schemes, a basic level of information about any schemes agreed between the NHS and manufacturers should be made public. This does not need to include commercially sensitive information but should indicate which outcomes are measured, the source of the data for the outcome metrics being used and how those outcomes are linked to price. This would help avoid unnecessary duplication of effort in the design of OBP schemes and inform conversations about the scale of the challenges in implementing OBP in the NHS.



**Recommendation:** GMHSCP, Government, NHS England, the pharmaceutical industry, NICE and all other relevant stakeholders should continue to explore the use of OBP schemes, with the aim of facilitating patient access to cancer medicines in cases where a simple discount on the medicine's list price cannot be agreed on a timely basis. Conversations should be taken forward on a joint basis, through forums and initiatives such as the Accelerated Access Collaborative.

**Recommendation:** GMHSCP, Cancer Research UK, NHS England, NICE and the pharmaceutical industry should work together to horizon scan medicines nearing regulatory submission which might be suitable for an OBP scheme. We believe such medicines would have the following characteristics:

- Potentially large benefit to patients receiving the medicine
- Small to moderately-sized patient populations
- Immature clinical trials data
- A disease profile where improvements in outcomes measurable in the short-term (including overall survival and non-progression/relapse) are particularly valuable.

**Recommendation:** NHS England or NICE should publish information on how outcomes are measured and linked to price in any OBP schemes for medicines in operation in the NHS. This should stop short of publishing commercially sensitive financial information.

## Which Outcomes Should Be Measured?

The use of OBP schemes could formalise the use of a broader range of outcomes than is currently systematically captured in the HTA process. It would also allow a medicine's price to be varied in the light of real-world evidence of its effectiveness in routine NHS use. Taken together, these factors could mean this price more closely aligns with the true value of that medicine to patients in an NHS setting (beyond clinical trials). Our research established the full scope of outcomes to be considered, as set out in Figure E3 below.

Through further engagement with patients and carers, a set of four outcomes (survival; disease progression, relapse or recurrence; long-term side effects; and return to normal activities) was identified as of greatest importance. We therefore recommend these four outcomes should form the "core" of any future OBP schemes negotiated by NHS England and pharmaceutical companies for cancer medicines, as set out in Figure E4 below.

NICE's HTA processes refer to all of these types of outcomes when deciding whether a new medicine should be reimbursed by the NHS, and the four outcomes listed will all affect whether NICE judges a medicine to be cost-effective. However, data on some of these outcomes would not currently be routinely captured in NHS clinical practice, particularly "long-term side effects" and "return to normal activities".

Our research has highlighted the importance of formally and explicitly using these outcomes when designing an OBP scheme for cancer medicines. Recognising these outcomes in future OBP schemes would ensure that real-world evidence of a drug's impact on these outcomes could be collected and used to align its price with the value it delivers to patients in the NHS, based on the outcomes that matter most to patients.

Figure E3: Outcomes “flower”

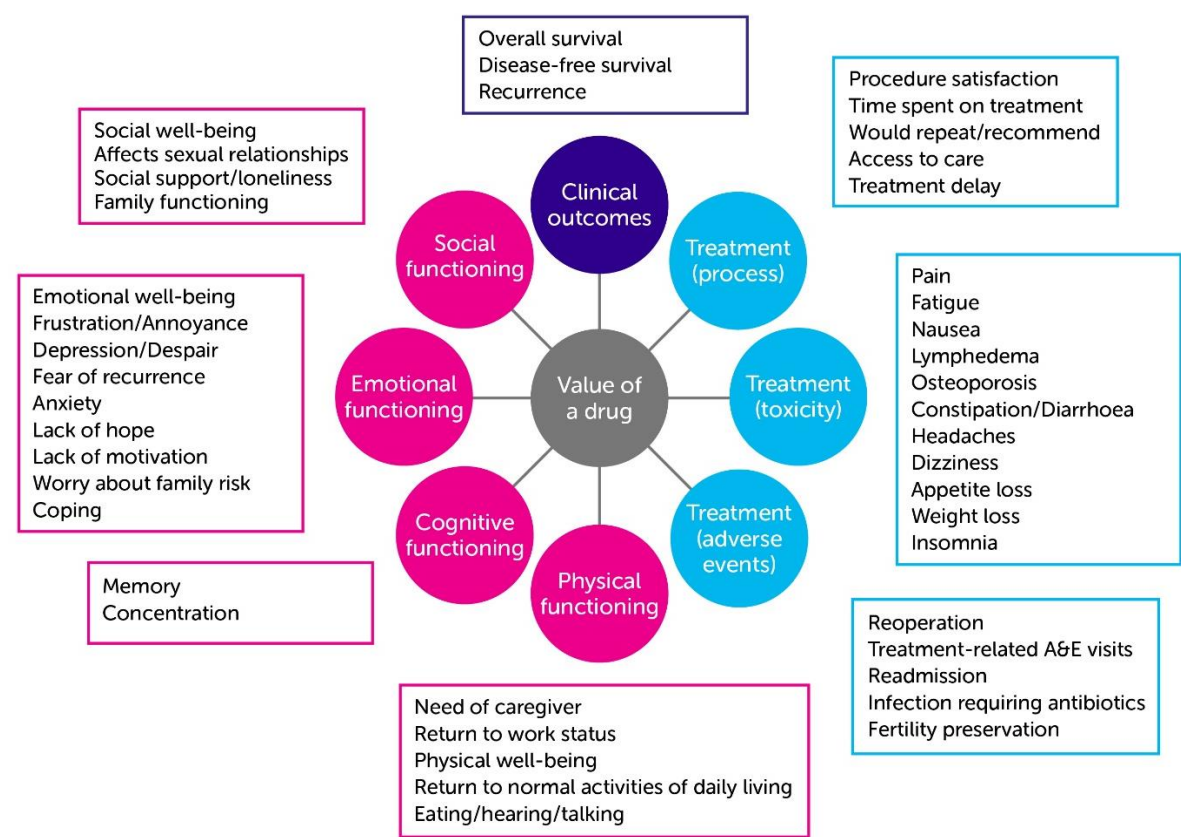
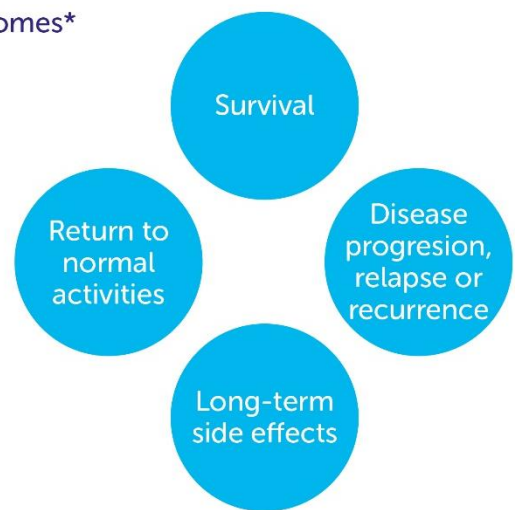


Figure E4: Outcomes framework

“Core” outcomes\*



Factors affecting the specific outcomes metrics chosen should include:

- Patient age
- Cancer type
- Cancer size and spread
- Intent to cure or manage disease

\*The treatment outcomes identified as the most important to people affected by cancer in our survey. We recommend the price the NHS pays for a drug under any future OBP scheme should be linked to NHS patient outcomes in these four areas.

Given a chosen set of outcome measures for a specific OBP scheme, there remains a need to understand the relative weights to be attached to those measures, and how the resulting composite measure of outcomes is linked to the price paid for the medicine. Options for achieving this should be explored in future research.

**Recommendation:** *As part of any future OBP schemes negotiated between NHS purchasers of cancer medicines and manufacturers, specific metrics should be included to measure the drug's effects on patients in the NHS, on the following four types of outcomes as standard:*

- *Survival*
- *Disease progression, relapse or recurrence*
- *Long-term side effects*
- *Return to normal activities*

**Recommendation:** *Future research into the use of OBP in the NHS should investigate the relative weights which should be attached to measures of the four “standard” outcomes (and potentially others) we wish to see included in future OBP schemes. This should include seeking the views of patients and other key stakeholders. This research should also clarify options for linking outcomes to a drug's price in practice.*

## Real-World Data Infrastructure

Real-world outcomes can be linked to price in a number of different ways, though our research has found that “binary” or “stepped” options (with a limited number of possible price points) are preferable to “continuous” schemes in order to minimise complexity. However, high-quality data on a drug's real-world benefits is needed to establish this link in the first place – although OBP schemes can also include the collection of additional trial data as well.

The cancer data infrastructure in England is already able to capture some of the “core” outcomes outlined above, including patient survival. However, these data are not always high-quality or complete. There also remains a need to explore to what extent data on other outcomes of importance (including long-term side effects and return to normal activities) are collected, where they are captured if so, and whether it is possible to link these with data on other outcomes in the way that would be required to operate an OBP scheme.

Determining how each outcome is measured for any given OBP scheme will need to consider the practicalities of data collection in the NHS with the current data infrastructure, and the need to avoid excessive administrative burden. A strong message from stakeholders interviewed is that, to succeed, OBP schemes need to be simple to operate.

**Recommendation:** *Future research into the use of OBP in the NHS should investigate with NHS staff the practicalities of collecting data for an OBP scheme, based on exemplar medicines and for measures of the four outcome types listed earlier.*

**Recommendation:** *As part of future research into the use of OBP in the NHS, a mapping exercise should be undertaken to ascertain the appropriate data sources, and identify “gaps” in the capacity to collect data on the “standard” outcomes specified above. This review should involve NHS Trusts providing cancer care, Public Health England, NHS England and the pharmaceutical industry.*

***Recommendation:*** NHS England and Public Health England should ensure resource is available within PHE to monitor and analyse in a timely manner the data submitted to SACT as part of any future OBP schemes adopted in the NHS nationally; and should explore the feasibility of using SACT or another consolidated database to capture all four “standard” outcomes, in order to facilitate their inclusion in future OBP schemes.