



# Making Outcome- Based Payment a Reality in the NHS

## *Phase 2: Practical Considerations*



November 2021

Together we will beat cancer

Greater  
Manchester  
Health and  
Social Care  
Partnership



CANCER  
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# Reference

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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 2020/2021, we committed £388 million towards cancer research.



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

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## Greater Manchester Health & Social Care Partnership

We formed the Greater Manchester Health and Social Care Partnership to oversee the devolution of our health and social care services. Our aim is to achieve the biggest, fastest improvement to the health and wellbeing of our region.

The Partnership is made up of our local NHS organisations and councils, as well as people from NHS England and NHS Improvement, our emergency services, the voluntary sector, Healthwatch and others including the mayor of Greater Manchester.



<https://www.gmhsc.org.uk/>

# Research Team

This research was conducted by:



# List of Acronyms

AAR	Accelerated Access Review
AWMSG	All Wales Medicines Strategy Group
CAG	Confidentiality Advisory Group
CDF	Cancer Drugs Fund
CPRD	Clinical Practice Research Datalink
DPA	Data Protection Act
EAMS	Early Access to Medicines Scheme
EMA	European Medicines Agency
EQ-5D	EuroQol Five-Dimensional instrument for measuring health-related quality of life
GMHSCP	Greater Manchester Health and Social Care Partnership
HES	Hospital Episode Statistics
HTA	Health Technology Assessment
LSIS	Life Sciences Industrial Strategy
MHRA	Medicines and Healthcare products Regulatory Agency
NCRAS	National Cancer Registration and Analysis Service
NHS	National Health Service
NHSE&I	NHS England and Improvement
NICE	National Institute for Health and Care Excellence
OBP	Outcome-Based Payment
OLS	Office for Life Sciences
ONS	Office of National Statistics
PHE	Public Health England
PROM	Patient-Reported Outcome Measure
QALY	Quality-Adjusted Life Year
RCT	Randomised Controlled Trial
RWE	Real-World Evidence
SACT	Systemic Anti-Cancer Therapy
SMC	Scottish Medicines Consortium

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# Foreword

Value and evidence in patient care play a key role in how the NHS in England commissions and funds cancer medicines. Yet, how and why we judge, measure and record the value of a new treatment is more than health economics and getting the most out of stretched healthcare budgets. It is also about ensuring the medicines the NHS commissions live up to the expectations of clinicians and patients and benefit people affected by cancer in the ways that matter most to them.

In the first phase of Cancer Research UK and Greater Manchester Health and Social Care Partnership's research programme into the use of outcome-based payment (OBP), we made the case that this more flexible way of paying for some cancer medicines could offer a range of benefits to the NHS, to manufacturers, and to patients. We concluded OBP could allow faster or more comprehensive patient access to some new medicines and offers a way to promote value for money from NHS budgets while rewarding genuine innovations that can demonstrate their benefits to patients in the NHS.

This is especially true in cases where there is uncertainty about a medicine's effectiveness and therefore the NHS would hesitate to reimburse the medicine's price in full until they have more evidence that it works in practice. In those cases, linking the reimbursed price of a medicine to the outcomes actually achieved in practice could mean patients have access to the medicine sooner than would otherwise be possible.

An OBP approach, reliant as it is on comprehensive and high-quality data collection on NHS patients' treatment outcomes, would also make capturing this data a system priority – generating valuable information on how that drug is benefitting (or not) patients. Indeed, since our first phase report was published, data from Public Health England's SACT dataset has played just this role, in supporting the successful re-appraisal by NICE of several medicines previously in the Cancer Drugs Fund.

But we think the NHS can go further. In the first phase of our research, we worked with people affected by cancer to understand the treatment outcomes they valued most. Among the outcomes they considered most important were 'clinical' outcomes like survival, but also outcomes focusing more on quality of life. We argued that to reflect the full value cancer patients get from their treatment, ideally future OBP schemes should include measures which capture a drug's impact across both clinical and quality of life factors – based on routine, at-scale, high-quality NHS data capture across all these outcomes.

We now seek to build on that work through this second phase of our research, which explores in greater detail the practical challenges to implementing such an OBP scheme in the NHS in England – in particular, the extent to which data on outcomes are already captured in the NHS, and whether this data is suitable to be used as part of an OBP scheme.

Our findings show that data collected through the SACT dataset may in some circumstances already be sufficient for an OBP scheme, based simply on outcomes such as survival and

treatment duration (as a proxy for disease progression) – although this conclusion is subject to key practical barriers noted in the report.

But we have also found that the NHS in England remains a way off the vision set out in our first phase report of being able to operate a national-level OBP scheme incorporating routinely collected data on patient quality of life outcomes. Capturing data on these outcomes is undoubtedly complex, and potentially resource intensive. If we are serious about ensuring patients are receiving the most beneficial interventions for them, and that these interventions are delivering the value they promise, we must find a way to make capturing and using data on quality of life as well as clinical outcomes an inherent part of practice.

Without building a system that can achieve this goal, we will not be able to implement a version of OBP which truly reflects a drug's entire potential benefits and value to patients. While practicality must be a key watchword in the move towards more flexible payment models, equally our ambition for the longer-term should not be limited by what is currently feasible. But this means investing in the workforce, data systems, and other solutions needed to overcome the barriers identified in this report.

The devastating and negative impact of COVID-19 on cancer services cannot be ignored in this conversation, and decision-makers' focus in the short to medium term is rightly on the recovery of cancer care and other services. Yet the NHS' response to COVID-19 also demonstrated it is capable of rapidly setting up new structures and collecting new data, when the need or the opportunity is great enough. It has also provided further proof of the potential for strong collaborative relationships between life science companies and the NHS, another underpinning enabler of OBP. As we seek to rebuild and eventually transform cancer services, we should not lose sight of tangential yet realistic opportunities to evolve existing processes for long-term benefits.

We believe the recommendations set out in this report – in particular those relating to investment in the NHS' data capabilities – are examples of such an opportunity. Moreover, these ambitions are aligned with the wider vision set out by Government of the UK as a life sciences hub, and making the most of data and technological solutions for patient benefit.

The NHS has an incredible opportunity to combine its emerging data capabilities and its enviable access to innovative new treatments, and to leverage the best possible evidence from both clinical research and clinical practice in improving patients' care. This opportunity will remain only partially realised if the NHS is unable to effectively and systematically track and link patients' quality of life outcomes with their 'clinical' outcomes. But achieving this will provide a powerful tool to support patients today, and in the future, to make decisions about their treatment that are right for them.

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# Executive Summary

## Outcome-based payment and the UK medicines landscape

Uncertainty about the effectiveness and cost-effectiveness of new medicines is becoming increasingly common, as is the corresponding risk that the NHS may pay for a medicine that in practice delivers insufficient benefit to justify its price. Factors contributing to this increasing uncertainty include:

- Differing patient demographics and treatment outcomes in clinical trials compared with clinical care in routine practice outside trials;
- Policy and process initiatives meant to promote earlier patient access to new medicines leading to health technology assessment (HTA) submissions with a less mature evidence base;
- Uncertainty about the long-term benefits (and adverse effects) for patients of highly innovative treatments that work in new and complex ways.

This uncertainty, combined with sometimes high list prices for some new treatment options, can make it harder for the NHS and manufacturers to agree a price which has an acceptable impact on NHS budgets and appropriately reflects the medicine's benefits for patients and innovation by its manufacturer. Difficulties in agreeing a medicine's price can delay or even restrict patient access to a new treatment option.

In England, when a new medicine is being considered for use on the NHS, its value for money – and clinical value for patients – is assessed through the National Institute for Health and Care Excellence (NICE)'s HTA process. This is accompanied by price negotiations between NHS England and Improvement's (NHSE&I) and the medicine's manufacturer. For oncology medicines in England, this challenge has been managed successfully since 2016 via the Cancer Drugs Fund (CDF)<sup>i</sup>. When the cost-effectiveness of the medicine is not initially clear from NICE's HTA process, the CDF permits patient access to a medicine at an agreed price while further clinical data are collected. This informs a later reassessment of the medicine, again through the NICE HTA process, to establish its benefits with more certainty and thereby to make a better-informed reimbursement decision.

Outcome-based payment (OBP) represents a related but alternative approach to the CDF. OBP seeks to directly manage the uncertainty over the medicine's effectiveness, by explicitly linking the price to the treatment outcomes achieved in patients. In this way, the price paid for the medicine directly reflects its performance according to expected, pre-agreed outcomes. The OBP arrangement is then part of the basis on which NICE determines, in its HTA process, whether the NHS should pay for the medicine. Under the CDF, the data collected can inform further future price negotiations between the NHS and the manufacturer, and so potentially affects the price the NHS pays for prescriptions of the medicine after its reassessment (if the NHS continues to fund its use). Under OBP however, the data are used to directly and dynamically adjust (or not) the payment made when the drug was prescribed.

OBP schemes are already in use in the UK, for cancer and non-cancer medicines. However, their

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<sup>i</sup> In July 2021, NHSE&I announced that the new Innovative Medicines Fund (IMF) will build on the success of the CDF to allow earlier access to a wider range of innovative medicines while the CDF will be guaranteed its current funding levels.

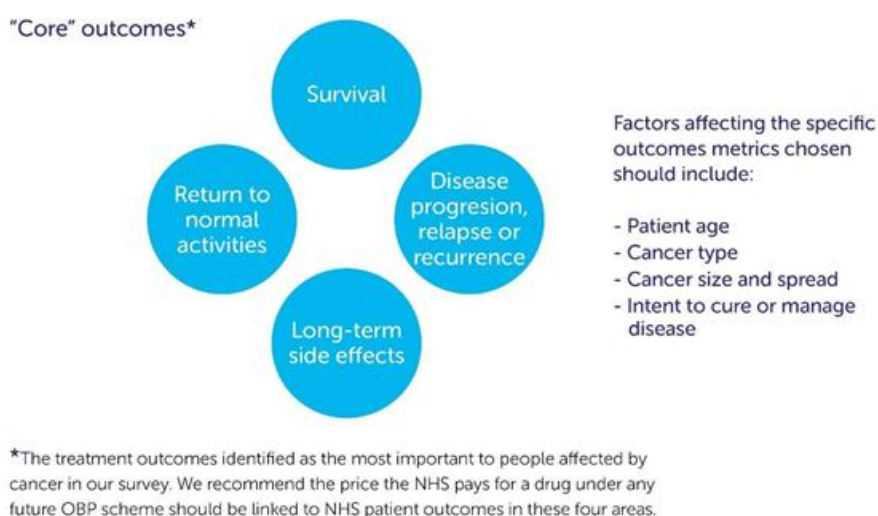
use is currently very limited, with concerns that more 'flexible' pricing schemes may impose extra administrative burden on the health service and its staff. But the idea of directly linking prices to patient outcomes in this way continues to attract interest, not only to resolve access challenges, but also to extract maximum value from limited NHS budgets, and to ensure the NHS has the data it needs to ensure the medicines it is funding are genuinely benefitting patients and meeting the expectations patients have for their care.

## Outcome-based payment is both desirable and feasible for some new cancer medicines

This report presents the second phase of research in Cancer Research UK and Greater Manchester Health and Social Care Partnership's '[Making Outcome-Based Payment a Reality in the NHS](#)' work programme, building on the findings in the phase 1 report, published in February 2019.<sup>1</sup>

The phase 1 report concluded that OBP may be both desirable and feasible for some new medicines, enabling faster and more comprehensive patient access while ensuring payment is aligned with effectiveness in the real world. It also identified a set of four outcomes of greatest importance to patients (see Figure 1 Core Outcomes), which were recommended as 'core' to future OBP schemes

Figure 1 Core Outcomes



This second phase of research explores the key practical challenges to overcome to successfully implement OBP in the NHS. Our research sought to identify existing NHS practices and infrastructure that offered opportunities to adopt an OBP approach now, as well as the longer-term changes needed to implement an OBP scheme fully in line with the vision set out in the phase 1 report. It focuses on specific arrangements for the NHS in England that can be implemented at a national level (and potentially at a local level).

## Key findings

### **The outlook on data availability for OBP in the NHS is positive but with some way still to go**

We undertook a ‘mapping’ exercise to identify existing measures (used in the NHS and more widely) of the four patient treatment outcomes from phase 1 (see Figure 1 Core Outcomes). This sought to understand the extent of existing NHS data capture on these key outcomes and whether these data are in a form suitable for a national-level OBP scheme. We demonstrate that data collection is advanced in some areas but requires investment in others.

The Systemic Anti-Cancer Therapy (SACT) dataset, now run by NHS Digital<sup>ii</sup>, already collects data on patient outcomes relating to (or serving as a proxy for) survival, disease progression, and (short-term) treatment side effects. A realistic and pragmatic local-level OBP scheme in the short-term could be based on survival, treatment duration and toxicity data captured through existing SACT data as well as electronic health records and e-prescribing systems. Data oversight and analysis could be led by NHS Digital.

However, in the long-term, an OBP scheme should also include data on returning to normal activities and long-term side effects via patient reported outcome measures (PROMs) data collection, and the data for disease progression would be optimised. The lack of a national dataset providing structured data on return to normal activities outcomes is a critical barrier which must be overcome for an OBP scheme incorporating these outcomes. NHS Digital and NHSE&I Cancer Quality-of-Life Metric Project may offer such an option soon, although the follow-up time of 18-months may limit its utility for an OBP scheme. Similarly, bespoke data collection arrangements would be needed as part of any national OBP scheme incorporating long-term side effects; Greater Manchester’s ePROMs initiative offers a local model of such arrangements.

### **Data linkage is needed to enable financial decisions**

In addition to collecting the necessary data in a format that can be used for OBP, the ability to clean the data and link these datasets to enable financial decisions (such as whether a rebate is due) needs to be considered.

Collection of clinical outcomes is relatively advanced and can now serve as a starting point for OBP schemes. However, data collection initiatives are evolving for ‘Long-term treatment side-effects’ and ‘Return to normal activities’ and could be developed in parallel, while data linkages between these and the other core outcomes are established through pilots and at scale. SACT data could in principle be linked with external datasets (once available) capturing PROMs data and specifically patients’ ability to return to normal activities whilst being treated with a medicine. Resource would also be needed to ensure a trusted third party (potentially NHS Digital) can collect PROMs data at scale and link these to individual-level patient data on clinical outcomes (including data already collected through SACT). Time lags due to different data collection timeframes across datasets may impact data linkage and will need to be considered.

In the short-term, an OBP scheme based solely on outcomes of a more clinical nature may be feasible. Investment is needed in optimising the quality and linkage of data from the Cancer Quality-of-Life Metric Project and introducing flexibility around the time points that data are

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<sup>ii</sup> At the time this research was conducted Public Health England was the body operating the SACT dataset, subsequently succeeded by NHS Digital following publication.

captured post-treatment.

Given the need for an OBP scheme to access, track, and link individual patient data across multiple datasets, a clear and robust framework for information governance is required. Obtaining explicit patient consent would be challenging, therefore an alternative legal basis may be required<sup>iii</sup>. Such approval should be achievable, provided the appropriate policies, procedures and safeguards are put in place. This is because – for the purpose of assessing patient outcomes – data cannot be completely anonymised; tracking and linking of data will be required at the patient-level, using a unique patient-level identifier to link different data sources (using a pseudonym). However, for patients to be supportive of an OBP scheme they must know, and have control over, exactly how and why their data are being used.

## **Operational practicalities must be overcome to implement OBP**

We conducted interviews with industry and system-level NHS stakeholders, and with NHS staff in local Trusts, to understand the other logistical and practical barriers such schemes would need to overcome.

Extra demands on hospitals where OBP schemes are in operation would need to be supported. These include financial costs, for example from additional data storage or setting up patient-reported outcome platforms (although some of these costs could be offset by any rebates for unsuccessful treatment, or via contributions from the medicines' manufacturers); as well as staff time (including clinical staff) associated with additional data collection and data cleaning. Investments into improving data quality and infrastructure are likely to provide additional patient benefit beyond the implementation of OBP schemes.

The rebate process will be complex to agree. NHS Trusts procure the medicines they prescribe; but completing and processing requests for rebates will impose a burden some individual Trusts may not have capacity to meet, so this might better be coordinated centrally by NHSE&I. Clarity is also needed on whether any rebates would remain with Trusts (which procure the medicines) or with NHSE&I (which ultimately pays for the medicine).

Given the practical challenges involved, implementing OBP will require buy-in from groups including clinical staff, Trust executives and national system stakeholders. Ensuring these groups are aware of why the underpinning data should be collected and the benefit to patients is essential. Buy-in from patients is also fundamental. Stakeholders engaged in our research – including national and Trust-level NHS staff, industry, and patients – had a mostly positive outlook on the practical feasibility of OBP despite the data and other practical barriers identified. In our focus group, patients were supportive of an OBP approach on the condition they would know, and have control over, exactly how and why their data are being used.

These practical considerations mean simplicity and transactability should be encouraged in any scheme design, though this should not detract from efforts to include data across all four outcome types. The optimal organisation and governance of OBP schemes, including data governance and the process by which any rebates might be paid by manufacturers to the NHS, should be investigated further at the national level, in conversation with Trusts and industry.

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<sup>iii</sup> Such as Section 251 approval

## Despite these barriers, OBP offers a ‘win’ for patients, NHS and industry






By modelling a hypothetical OBP scheme using retrospective (population-level) NHS patient outcome data, we undertook initial testing of the possible financial outcomes from the use of OBP compared to a simple pricing arrangement. We conclude that OBP seems likely to reduce the financial risk to the NHS caused by clinical uncertainty, though this is sensitive to the outcomes included in the scheme, the size of the rebate, and the ‘successful’ treatment price level.

OBP offers a ‘win’ for patients, in cases where simple pricing approaches would mean delayed or restricted patient access. Expediting the pricing agreement process consequently benefits the NHS and industry. As noted above, the CDF already does something similar by offering patient access over a period of further data collection. Yet, when uncertainty remains about a medicine’s clinical effectiveness and no further data are expected, OBP could offer a route to faster or more comprehensive patient access whilst also providing further data.

### Key characteristics for design of an OBP scheme

Through engagement with our expert Steering Group and a simulated case study of an OBP scheme in the NHS, we have narrowed down what an OBP scheme in the NHS might look like in practice. We have identified a plausible set of ‘default’ characteristics for a scheme (outlined below), to be adapted as appropriate in individual cases, recognising the importance of simplicity and practicality. The characteristics of any OBP scheme should be agreed between the NHS and the drug’s manufacturer as part of the commercial negotiation process.

A pragmatic default set of characteristics for future OBP schemes could specify the following:

-  Prices and rebates are linked to treatment outcomes for individual patients, rather than average outcomes across populations
-  A two-level price, with an initial payment by the NHS and a subsequent rebate to a pre-agreed lower price level if the pre-agreed, expected ‘success’ levels of patient outcomes are not met
-  ‘Successful’ treatment is defined as where patient outcomes meet all of the separate threshold levels set for each of the individual outcome measures
-  Outcome ‘thresholds’ indicative of treatment success can be based on available clinical trial data and available real-world-data for an outcome metric, but should also align with points identified in NICE’s HTA process that determine cost and clinical effectiveness
-  The scheme should use all four outcomes types identified in Phase 1, with omissions agreed between all parties on a case by case basis. Not already having NHS data readily available should not on its own justify omitting an outcome type

NHSE&I should recognise the specific value of OBP in cases where data collection from completed clinical trials or managed access arrangements has failed (or is unlikely) to resolve clinical and cost uncertainties.

## Key conclusions and recommendations

Phase 2 identified key practical barriers and enablers to implementing OBP in the NHS. Through a range of investigative techniques and analyses we have:

- Provided greater clarity on data available now and potentially in the near future to support an OBP scheme;
- Provided a clearer understanding of operational issues and burdens associated with the implementation of OBP schemes;
- Modelled the potential financial impact of an OBP scheme to the NHS, demonstrating that OBP can reduce the risk to payers from uncertainty about the cost-effectiveness of cancer medicines;
- Identified a pragmatic set of characteristics for an OBP scheme.

With further investment by NHSE&I in data capabilities and stakeholder support, the challenges listed in the recommendations are achievable. The 'ideal' OBP scheme proposed in phase 1 would consequently be conceivable in the long-term but pragmatism dictates a simpler approach be pursued at least initially.

The advancement of electronic health records and national datasets, as well as improved data linkage opportunities, mean that data on survival and disease progression are mostly available and can be leveraged for an OBP scheme. However, the landscape for collecting patient-reported outcomes is evolving, capturing data on long-term side-effects and return to normal activities of daily life is a work in progress. In the short-term, an OBP scheme based solely on outcomes of a more clinical nature may be feasible, however it is imperative to capture the quality-of-life outcomes in future OBP schemes to reflect the drug's full value to patients.

In addition to the data requirements (availability, quality, linkage, delays, governance), there are significant operational practicalities which need to be considered or improved to be able to implement an OBP scheme:

- expanded mechanisms are needed to collect patient outcome data in a usable format;
- the time and cost burden of OBP needs to be recognised;
- details of the rebate process need to be clarified;
- the need to achieve buy-in from NHS staff and organisations;
- the need for patients to buy-in to their data being used for OBP;
- a clear and robust framework for information governance is required.

Although it is not currently possible to 'fully' pilot an OBP scheme with a complete dataset for each of the four core outcomes, data availability and linkage should not be considered static. As the data landscape is changing, pilots capturing just clinical outcomes or all core outcomes, including the less developed ones, provide a valuable learning opportunity to inform and implement a more complex OBP scheme in the future.

## Piloting a local OBP scheme

A pilot study with available data could offer a 'proof of concept' of the model developed during phase 2 and provides the opportunity to 'test' key research questions encompassing operational and practical issues related to governance, data quality, data linkage, consent, capacity and cost etc (fully described in section 4.5 Piloting an OBP scheme).

With clear success criteria, the pilot would provide further evidence to understand whether a national OBP scheme would be viable. Implementing the recommendations below would create an opportunity for an OBP scheme to be more comprehensively tested at a national level (if deemed necessary and viable), and to further the evidence base.

## Policy recommendations

The below recommendations would support the implementation of more complex OBP schemes in the long- term if this were to be taken forward nationally.

1. A local-level pilot of an OBP scheme based on data available for clinical outcomes (survival, disease progression, and short-term treatment side effects) would be feasible and desirable. Relevant stakeholders should convene to discuss and agree how a pilot could be taken forward.
2. NHSE&I should recognise the potential benefits of OBP. This includes (i) the potential for patients to have access to some new medicines sooner than with a simpler pricing scheme (ii) and ensuring that the NHS pays only for the outcomes actually achieved for patients. Data collection should be encouraged for this purpose.
3. To minimise data barriers to an OBP scheme of the kind set out in our phase 1 research study, NHSE&I and NHS Digital should invest to:
  - Optimise data collection to more precisely capture disease progression/ relapse/recurrence in the SACT dataset.
  - Allow patients to report long-term side effects and their ability to return to normal activities through PROMs data collection infrastructure (for example via online patient portals).
  - Optimise the quality and linkage of data from the Cancer Quality-of-Life Metric Project and introducing flexibility around the time points that data are captured post-treatment.
4. Where OBP schemes are implemented, dedicated resources should be made available to support the underpinning data collection. This could include:
  - Funding for local NHS Trusts to employ data teams, and for NHS Digital to support the quality and completeness of Trust-level data collection (and analysis of that data).
  - Resource to ensure a trusted third party (potentially NHS Digital) can collect patient-reported outcomes data at scale and link these to individual-level patient data on clinical outcomes (including data already collected through SACT).
5. The Department of Health and Social Care, in conversation with Trusts and industry, should further investigate the optimal organisation and governance of OBP schemes, including data governance and the process by which any rebates might be paid by manufacturers to the NHS.

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# Introduction

Previous work on ‘Making outcome-based payment (OBP) a reality in the NHS’<sup>1</sup> (Phase 1) demonstrated that OBP can be a suitable mechanism for tackling the challenges posed by some innovative cancer therapies which manifest as delayed access to potentially life-extending and life-improving treatments. The previous project also identified the key outcomes against which the success of cancer medicines should be judged, based on what really matters to cancer patients. However, the research highlighted some challenges and questions around the optimal implementation of OBP for cancer medicines, namely:

- What existing data sources can capture the outcomes of interest, and what are the gaps?
- How should outcomes be linked with payments in the optimal design of an OBP scheme?
- What are the practical challenges for implementing OBP in the NHS, and how could they be overcome?

Through the research programme described in this report, we sought to address these questions through a range of methods: an interrogation of the literature and in-depth assessment of data availability; consensus workshops with key stakeholders and experts; interviews with NHS trust staff, national bodies and industry on operational practicalities; and a focus group with patients. Finally, we sought to test the feasibility and potential impact of OBP on patients, commissioners, and manufacturers, through a hypothetical simulation of an OBP scheme. By providing insight into how OBP could be implemented in the NHS for cancer medicines in England, and the potential benefits and costs of doing so, we provide further clarity on how and why OBP could offer an important route for patient access to some innovative medicines.

In this introduction, we set out the scope for novel pricing schemes to overcome current challenges in patient access (1.1), and describe in more detail the findings of – and questions raised by – the Phase 1 research (1.2), before presenting an overview of the methods that we employed to move forward testing the case for OBP (1.3).

## 1.1 Pricing schemes to overcome challenges to patient access

Around 1,000 people are diagnosed with cancer every day in the UK, with a significant proportion of those (in England around 28%) receiving cancer medicines as part of their primary treatment.<sup>2,3</sup> The drug development pipeline continues to deliver new treatment options, with the number of cancer medicines in late-stage development expanding by 19% in 2018 alone, and 63% over the previous five years.<sup>4</sup>

Regulators and health technology assessment (HTA) bodies such as the National Institute for Health and Care Excellence (NICE) seek to ensure that patients are granted access to medicines that are safe and effective, and represent an efficient use of limited health care resources. These decisions are based on the best available evidence of patient outcomes, usually largely drawn from randomised controlled trials (RCTs). However, as the nature of drug development evolves, the challenges in assessing new medicines’ clinical and cost effectiveness are also

evolving.

Increasingly innovative treatment options such as immunotherapies, cell therapies and precision medicines are being developed. These may offer a new way of treating a particular cancer type, which interacts with the biology of the disease in a novel way. They may target smaller sub-groups of patients whose tumours express specific genetic characteristics. In some cases, they may even offer a potentially curative option for disease which could previously only be managed as a chronic condition.

While there is huge potential for patients to benefit from these advances, their innovative nature can make it harder for health systems to assess with confidence what the treatment outcomes of patients receiving these medicines are likely to be (especially in the long-term), based only on the data available from clinical trials. Regulators and HTA bodies are therefore faced with greater uncertainty around these new medicines' clinical and cost effectiveness when making approval decisions.

These challenges are exacerbated by the commendable efforts from regulators and policymakers to enable earlier access to medicines through process reforms and policy initiatives. This can lead to approval decisions being made based on less mature clinical trial data and 'surrogate' (rather than direct) measures of a medicine's benefits to patients – again increasing the uncertainty facing regulators and HTA bodies. However, delaying patient access while waiting for the evidence base to mature and uncertainty to reduce means patients miss out on the opportunity to benefit from potentially life extending and/or life improving new treatments.

In addition, there is often further uncertainty about how a treatment will perform in clinical practice (outside the context of a clinical trial) because of differences in the mix of patients receiving the medicines in these two settings. RCTs often exclude patients who are older or who have major comorbidities; trial participants may also show higher treatment adherence than patients in clinical practice. In smaller patient samples it is not always possible to conduct a full RCT and therefore real-world evidence from an OBP scheme will not in all cases substitute RCT data. However clinical trial data are imperative when evaluating the clinical and cost-effectiveness of medicines, and there are inherent limitations when using real-world evidence. The RCT is the most scientifically rigorous method of hypothesis testing and is regarded as the gold standard for evaluating the effectiveness of interventions<sup>5</sup>, whereby confounding variables can be controlled for.

This potential for misalignment between patients' outcomes in trials and the 'real world', and uncertainty about long-term patient outcomes in particular, creates financial risk for both the payer and the manufacturer – the risk of either 'overpaying' if a medicine fails to deliver the expected benefits, or of 'undervaluing' genuine innovations that deliver significant benefits to patients.

This is especially so given the global trend for many new medicines to be marketed at increasingly high list prices,<sup>6</sup> particularly in cancer.<sup>7,8</sup> Some argue that these increases may be disproportionate to the added value that clinical trials data (particularly if immature) suggest the new medicines can offer over other treatment options. For example, Salas-Vega et al.<sup>8</sup> demonstrate that cancer drug treatment costs are high and only weakly associated with clinical benefits, especially in the US but also the UK. However, it should be noted that such analyses only consider list price, and therefore do not capture the (commercially sensitive) discounts that are successfully obtained by NHSE&I, which can be substantial<sup>9</sup>

In this context, the collection and assessment of real-world evidence (RWE) is increasingly recognised as a necessary complement to clinical trials data in understanding a medicine's effectiveness in clinical practice and its value for money in health systems with limited (and often stretched) budgets. The formal incorporation of RWE into new medicines approval processes is becoming more common – including in England through the post-2016 Cancer Drugs Fund (CDF).

But the way new cancer drugs are paid for around the world – and in the UK in particular – has also changed to reflect this context of high cost and high uncertainty, with the growing use of patient access schemes. In the UK, many of these schemes take the form of a simple percentage discount to a medicine's list price, used to offset the risk and uncertainty noted above to the NHS (the payer in the UK system; the UK context is examined in more detail in Chapter 2 below). Nonetheless, this financial risk remains a key challenge in delivering swift and comprehensive access to these new treatment options for patients.

In response, researchers and policymakers are continuing to look to new, more innovative pricing models, including more explicitly linking the price paid for a medicine with the treatment outcomes of the patients who receive the medicine. This approach is known as outcome-based payment (OBP). Under OBP, medicines that perform as expected and deliver pre-agreed outcomes are ultimately reimbursed at a pre-agreed price, while medicines that do not deliver on these outcomes are ultimately reimbursed at a pre-agreed lower price or not at all.

This flexibility means OBP offers a way to manage the financial risk to manufacturers and payers noted above of either undervaluing or overpaying for a new drug – and so mitigates this barrier to patient access to new medicines. Finding a way to link a medicine's price with patient outcomes without unnecessary burden to the health service and patients, is therefore an important challenge to overcome – and is the aim of this research.

## 1.2 Building on Phase 1 research

In this report we describe the progress made in the second phase of research, undertaken from autumn 2019 to autumn 2020, for the project '[Making outcome-based payment a reality in the NHS](#)'. Phase 2, reported here, builds directly on Phase 1 of the research, which was carried out during 2018 and reported in February 2019.<sup>1</sup>

Phase 1 sought to establish the feasibility of an OBP approach within the NHS in England and explore which patient outcomes should be included in such schemes. The research for Phase 1 included a review of the published literature on OBP-like schemes for medicines internationally; a review of literature on measures used to assess patients' outcomes from cancer treatment; and 13 interviews with NHS clinicians and commissioners of cancer services in England, the pharmaceutical industry and academia. The research also included two focus groups with people with lived experience of cancer treatment and a survey (with 164 respondents) of people with lived experience of cancer. The focus groups and survey – which built on an initial literature review – aimed to identify which outcomes from cancer treatment are most important to link to the amount the NHS pays for them, in the view of the people who receive the treatments and of the people who care for them.

From the research in Phase 1, we found that there are many examples internationally of schemes linking the amount paid for a medicine (for a wide range of diseases) to the outcomes

achieved. However, nearly all such schemes rely on measuring clinical outcomes (like survival), rather than patient reported outcomes (in particular those relating to patient quality of life). Furthermore, the existing schemes rely on a single clinical outcome in any given payment scheme, when determining whether the medicine had treated patients successfully, rather than including a number of different dimensions of outcomes.

It was concluded on the basis of the Phase 1 research that an OBP approach for some, but not all, new cancer medicines would be both desirable – as a way to promote faster or more comprehensive patient access to these medicines – and potentially feasible in the NHS in England. However, the research also outlined challenges to the implementation of OBP, not least the technical challenge and resource burden of collecting timely, complete and accurate data about patients’ outcomes.

Bearing these challenges in mind, the Phase 1 research considered OBP to be most suitable for new cancer medicines with the following characteristics:

- Where there are early and promising clinical trial data about likely outcomes, but where significant uncertainties remain about the outcomes that would be achieved in routine clinical practice for people treated in the NHS;
- When the NHS and the medicine’s manufacturer therefore find it difficult to agree a fixed, discounted price for the medicine;
- Where improvements in outcomes can be measured within a year or two of treatment; and
- When the patient population to be treated is small to mid-sized rather than large<sup>iv</sup>.

The focus groups and patient/carer survey showed that there were four types of treatment outcomes that were, as a group, deemed to be considerably more important than any others for inclusion in OBP schemes for cancer medicines, namely: survival; progression, relapse or recurrence; long-term (post-treatment) side effects of the treatment; and return to normal activities of daily life (Figure 1 Core Outcomes).<sup>10</sup>

The report of Phase 1 contained eight recommendations, the first of which was for all stakeholders to “continue to explore the use of OBP schemes” – see Box 1. Phase 2 of the research was commissioned by Cancer Research UK and the Greater Manchester Health and Social Care Partnership (GMHSCP) to provide further evidence in support of that and to help to address recommendations 4-7 (Box 1). The remaining recommendations (2, 3 and 8) continue to apply. The specific aims and objectives of Phase 2, and how that research was carried out, are described in section 1.3.

### Box 1 – Recommendations from Phase 1

1. GMHSCP, Government, NHSE&I, the pharmaceutical industry, NICE (the National Institute for Health and Care Excellence) 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.

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<sup>iv</sup> Medicines with larger patient populations indicated are likely to have less uncertain outcomes after clinical trials, ceteris paribus, and so are less likely to need OBP rather than a simpler pricing arrangement. Medicines with very small patient populations may not be worth incurring the fixed costs of setting up data collection and rebate arrangements for them.

2. GMHSCP, Cancer Research UK, NHSE&I, 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:
  - a. Potentially large benefit to patients receiving the medicine
  - b. Small to moderately-sized patient populations
  - c. Immature clinical trials data
  - d. A disease profile where improvements in outcomes measurable in the short-term (including overall survival and non-progression/relapse) are particularly valuable.
3. NHSE&I 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.
4. 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:
  - a. Survival
  - b. Disease progression, relapse or recurrence
  - c. Long-term treatment side effects
  - d. Return to normal activities
5. 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.
6. 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.
7. 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, NHSE&I and the pharmaceutical industry.
8. NHSE&I and Public Health England (PHE) should ensure resource is available within PHE to monitor and analyse in a timely manner the data submitted to Systemic Anti-Cancer Therapy (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.

## 1.3 Phase 2 aims and methods

Figure 2 sets out the objectives of Phase 2, and also outlines the main method of investigation used to meet each objective.

### Figure 2 Outline of Phase 2 objectives and methods

**1. Identify data:** identify what data are available within the NHS to support an OBP scheme based on the outcomes framework developed in Phase 1, and understand the options and feasibility for collecting further data as required for the scheme's operation.

*Rapid evidence assessment; Interviews*

**2. Map to medicines & cancer types:** Identify classes of medicine and/or cancer types for which these existing NHS data could most meaningfully function as relevant clinical and patient-reported outcomes.

*Interviews; Horizon scanning*

**3. Develop a scheme and understand operational practicalities:** Understand the practicalities of operating an OBP scheme and develop theoretical models for how prices could be linked to outcomes (including the relative weighting of different patient outcomes).

*Workshops; Interviews*

**4. Test the scheme:** Conduct a preliminary retrospective analysis and theoretical testing of an OBP scheme based on the principles outlined in Phase 1.

*Simulation modelling*

### **5. Recommend a way forward**

Use the insights developed to produce recommendations for the design of the proposed final pilot phase of the research programme (Phase 3)

The stages of qualitative and quantitative work are outlined in more detail in Box 2.

## Box 2 – Phase 2 methods

The research consisted of the following stages:

- 1. Project Initiation Meeting.** An inception meeting with the project Steering Group was held to agree research methods and draw on Group members' knowledge of relevant literature and data. In all, a total of five meetings of the Steering Group were held across the project's duration. The Steering Group comprised members from major stakeholders, many of whom had also participated in the Steering Group during the Phase 1 research. Group members were drawn from: Cancer Research UK; GMHSCP; the Department of Health and Social Care (DHSC); NHS England and Improvement (NHSE&I); the National Institute for Health and Care Excellence (NICE); the Office for Life Sciences (OLS); Public Health England (PHE); patient representatives; clinicians; academia; and the pharmaceutical industry. Individuals' participation in the Steering Group was as subject experts rather than representatives of their respective organisations. Ethical approval (for the interviews and focus groups – see below) was provided by the University College London Research Ethics Committee.
- 2. Outcome measurement and data assessment.** A rapid evidence assessment of the literature identified the available instruments and metrics that align with the four outcome areas: survival; disease progression, relapse or recurrence; long-term treatment side effects; and return to normal activities of daily life. This was supplemented by the knowledge of members of the research team, based on other work being undertaken by the University of Manchester; and by interviews with key individuals (patients, oncologists, cancer pharmacists, and representatives of the Cancer Drugs Fund). The results of this stage are presented in section 3.1 of the report.
- 3. Identifying candidate medicines.** Interviews were held with five clinical advisers and two patients from the Steering Group to identify which categories of medicines might be suitable for OBP schemes. In parallel, we looked at cancer medicines recently launched (in the UK) and horizon-scanned a commercial medicines pipeline database. For recently launched medicines, we collected information on all NICE appraisals for cancer medicines between January 2015 and December 2019 and identified several which might have been suitable for OBP. Reviewing the pipeline database revealed well over 100 candidates which met key characteristics to be considered suitable for an OBP scheme.
- 4. Workshops and interviews to investigate operational practicalities.** A consensus workshop was held with the Steering Group to agree the characteristics of a theoretical OBP scheme to be applied and tested. A second workshop discussion was held with the Steering Group in June 2020 to agree the principles used to define 'successful' treatment in the context of such a scheme. The results of these workshops are presented in section 3.2. Between April and August 2020 (including a pause for several weeks during a severe period of Covid-19 pandemic pressure on NHS staff) we undertook a programme of 12 semi-structured interviews with operational staff in four NHS Trusts delivering cancer services, plus six interviews with

key staff in national bodies – NHS Digital, NHSE&I, NHSX, NICE, PHE – and three interviews with pharmaceutical industry managers. The focus of the interviews was on the practicalities of collecting and collating outcomes data and using it to determine outcome-based payments. A focus group interview with patients was also conducted to understand patient experiences of data collection and their views on the potential burden on reporting outcomes data required for an OBP scheme. The interviews and focus group are reported in section 3.3.

5. **Simulation modelling of an OBP scheme.** Owing to the unavailability of linked outcomes data across the four outcome types, it was not possible to conduct a retrospective quantitative analysis of how an OBP scheme might have looked had it been applied to a recently launched cancer medicine. Instead, the research team used a simulation model to theoretically test the financial impacts on NHS payers and on the manufacturer of a hypothetical OBP scheme. The model used a combination of assumptions and published clinical survival and treatment progression data for a recently launched cancer medicine in use in the NHS. The simulation and its results are explained in section 3.4.



## 2 Background: Funding medicines based on their outcomes in patients

The key premise of OBP is to link payment for cancer medicines with the outcomes that those medicines help patients to achieve in practice. This is not a novel concept. Indeed, the whole premise of HTA – and the health economic evaluation that forms a key part of the assessment – is to ensure that health technologies achieve what they intend to: to address a health problem and to improve quality of life. However, the evidence needed to support these assessments of new medicines is not always sufficient to make a definitive decision. OBP represents one way to address this problem without delaying patient access to the new medicines, and at the same time reassuring patients and NHS staff that they are getting the treatment benefits the NHS is paying for. In this section we set the scene for our research and its findings by briefly outlining how reimbursement decisions are currently made (2.1), the current options for providing access to cancer medicines when there is significant uncertainty around their effectiveness or cost-effectiveness (2.2), and finally what the shortcomings of this funding landscape are, to which OBP could be a solution (2.3).

### 2.1 The role of patient outcomes in reimbursement decisions

HTA refers to the systematic evaluation of properties, effects or impacts of a health technology, a key part of which is an economic evaluation which compares the new treatment under assessment to current practice in terms of its impact on overall health outcomes (clinical-effectiveness) and costs (cost-effectiveness), based on available data (usually from clinical trials). HTA is conducted by NICE in England (whose decisions also usually apply in Wales and Northern Ireland) and by the SMC in Scotland.

In order to assess the cost effectiveness of a new treatment, outcomes are measured in terms of quality-adjusted life-years (QALYs), which combine length of life with health-related quality of life measured using the EQ-5D instrument.<sup>11</sup> NICE compares the cost effectiveness of a new treatment with a threshold of £20,000-30,000 per QALY gained (with the health benefits gained from some medicines that meets its 'end of life' criteria receiving an additional weighting, in practice raising the threshold to around £50,000 per QALY gained for those medicines). Below this threshold, a treatment is considered to offer good value for money, and therefore a worthwhile investment of NHS resources. In parallel with the HTA process, the medicine's manufacturer and NHSE&I can conduct a commercial negotiation with the aim of agreeing a price for the drug at which it meets this cost effectiveness threshold and has an acceptable impact on NHS budgets. Patients' treatment outcomes – as captured by the QALY – are therefore already an integral part of access and reimbursement decision-making in England.

The work described in this report as well as the Phase 1 report seeks to go further, and outlines

how the collection and use of outcomes data that capture quantity and quality of life can further be used to tie payment to patient outcomes more explicitly – specifically, linking a medicine’s price to the outcomes of patients receiving the drug in the NHS (rather than in a clinical trial, as is usually the case in the HTA process). This may be particularly helpful in cases where the decision to reimburse a health technology is subject to significant uncertainty which, as outlined in section 1.1, is becoming increasingly relevant.

## 2.2 Funding of medicines under uncertainty: what are the current options?

There are currently two main (and potentially overlapping) options available in England for funding cancer medicines under uncertainty: coverage with evidence development and flexible pricing schemes.

The CDF was reformed in 2016 to provide access to new cancer medicines where clinical trial data are sufficiently promising to suggest that the drug plausibly offers value for money, but where there is uncertainty about its clinical benefits, and the manufacturer and NHSE&I are unable to agree a price at which NICE is confident the drug is cost effective. Access is provided while further evidence is collected to address this uncertainty, both from ongoing clinical trials and from the medicine’s use in the NHS. The main source of NHS data is usually NHS Digital’s SACT (Systemic Anti-Cancer Therapy) dataset. At the end of a medicine’s managed access period, the new data are considered by NICE, which makes a final recommendation on whether the drug is clinically and cost effective. This may also include further commercial negotiation between the manufacturer and NHSE&I, to agree a new price for the medicine that reflects this updated data.

The CDF thereby provides ‘coverage with evidence development’<sup>12</sup>, as defined in our Phase 1 report. The CDF has contributed significantly to improvements in cancer patients’ access to new medicines in England since 2016. By March 2020, the new CDF had allowed, through 40 Managed Access Agreements (MAAs), over 26,700 cancer patients to receive innovative treatments they would otherwise have been unable to access.<sup>13</sup>

As noted in section 2.1, most new cancer medicines, whether they are recommended for routine commissioning or by the CDF, are approved on the basis of a pricing agreement between the manufacturer and NHSE&I following commercial negotiation. In most cases, this will take the form of a confidential simple (i.e. percentage) discount on the medicine’s list price. This is an important part of dealing with uncertain cost-effectiveness by reducing the range of plausible cost-per-QALY estimates produced by NICE.

However, as noted in section 1.1, researchers, industry and policymakers globally are all exploring options for more flexible and innovative pricing arrangements, going beyond a simple discount. In the UK context, the 2016 Accelerated Access Review and the 2017 Life Sciences Industrial Strategy both recommended that NHSE&I adopt more flexible pricing arrangements, including conditional reimbursement and outcome-based schemes;<sup>14,15</sup> and the 2019-2023 Voluntary Scheme on Branded Medicines Pricing and Access,<sup>16</sup> agreed between Government, NHSE&I and the pharmaceutical industry, committed to increasing commercial flexibilities for companies whose products offer significant value for the NHS.

More recently, NHSE&I has released the NHS ‘Commercial Framework’, which sets out the principles on which NHS commercial medicines activity will be based. Among other things, it

outlines the commercial flexibilities NHSE&I can make available and the circumstances where these could be considered.<sup>17</sup> Flexible pricing approaches can be supported either through routine commissioning or through the CDF model.

This indicates a willingness, in principle, to work more flexibly with stakeholders to secure prompt patient access and fair reimbursement for medicines; and the draft Commercial Framework notes that the “increased availability of confidential commercial flexibilities is expected to be beneficial for patients, the NHS, individual pharmaceutical companies and the life sciences sector more broadly”.

However, the draft Commercial Framework is also wary of the additional administrative burden such flexibilities may impose on the health service and those who work within it. The Commercial Framework sets an expectation that simple discounts must be “fully demonstrated to be unsuitable” before more flexible and complex arrangements can be considered.

The NHS in England thus has established approaches to dealing with risk and uncertainty in decisions on the funding of new cancer medicines, and these approaches are continuing to evolve.

## 2.3A place for OBP in England’s access landscape

Our Phase 1 research concluded that OBP is an important tool that the NHS should look to develop and utilise as part of this evolving context. Although it will not be appropriate for every new medicine, OBP can offer the NHS a valuable extra option in responding to the trends in the regulatory and drug development landscape outlined in the previous chapter.

A small number of national OBP schemes have previously been implemented in the UK, for example to support the introduction of Velcade (bortezomib) for treating relapsed multiple myeloma, and hepatitis C treatment Olysio (simeprevir) (see Phase 1 report for further details).<sup>1</sup> However, the use of OBP schemes is still limited. Whilst there are examples of smaller regional OBP schemes<sup>v</sup>, they are not always open to scrutiny due to their commercial in confidence nature, which means that there is less opportunity to learn from them.

To demonstrate the added value that OBP could offer, it is worth stating more explicitly the differences between OBP and the CDF. As noted above, NICE can recommend plausibly cost effective drugs for the CDF for a time-limited period, during which further evidence on their effectiveness is collected from ongoing clinical trials and from their use in the NHS. These extra data are used to inform an HTA re-appraisal of the medicine by NICE and a final recommendation on whether NHSE&I should continue to fund the medicine’s use.

OBP represents a related but alternative approach. Rather than looking to resolve the uncertainty about a medicine’s cost effectiveness through further data collection and a more precise reassessment of its clinical benefits without agreeing explicitly how these benefits will be reflected in the medicine’s price, OBP seeks to manage that uncertainty while real world data on patient outcomes are collected, by directly linking a medicine’s price to the treatment outcomes of the patients who receive the medicine in the NHS.

Under the CDF, the data collected can inform further, future price negotiations between the

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<sup>v</sup> For example see page 27 of this Greater Manchester Combined Authority Report: [https://www.gmhsc.org.uk/wp-content/uploads/2019/04/GM-Cancer-Annual-Report-2018\\_19-Finalversion\\_march19.pdf](https://www.gmhsc.org.uk/wp-content/uploads/2019/04/GM-Cancer-Annual-Report-2018_19-Finalversion_march19.pdf)

NHS and the manufacturer, and so potentially affect the price the NHS pays for prescriptions of the medicine after its reassessment (if the NHS continues to fund its use). Under OBP however, the data are used to directly adjust (or not) the payment made when the drug was prescribed.

As noted in section 1.2, our Phase 1 report set out possible characteristics which might make a new cancer medicine particularly suitable for an OBP approach. Some of these criteria are also reflective of characteristics of medicines for which the associated clinical and financial uncertainty at the point of HTA is already being successfully managed through the CDF to deliver faster or more comprehensive patient access. However, given the emphasis placed (to date) on additional data collection from ongoing clinical trials as part of the planned evidence development for medicines entering the CDF, there may be a complementary role for OBP in cases where uncertainty remains about a drug's clinical effectiveness even once clinical trial data are mature (i.e. no further data collection is expected from the trial or any further data collection would not be expected to help resolve that uncertainty).

These might include cases where a drug uses a mechanism of action not previously used in the indication; where a small population size makes a traditional RCT unfeasible; or where the clinical or demographic characteristics (e.g. sex, race or age) of the NHS patient population were not well-represented in the clinical trial. In such cases, a more direct approach to managing the financial risk (rather than seeking to resolve this clinical uncertainty within the desirable timeframe for patient access) may be appropriate. OBP can provide such an approach – but only if high-quality data can be captured at scale within the NHS, in a reasonable timeframe, on the clinical and/or quality of life outcomes of NHS patients receiving the drug.

OBP can be seen as an attractive option for reasons that go beyond simply the potential for faster and/or more comprehensive patient access to a new treatment. The NHS operates to a fixed budget, and OBP offers a way to ensure that where the expected value is not being derived from its spending on specific medicines, then that outlay can be recouped and reinvested in other health services.

An OBP approach encapsulates the desire that the NHS should seek to ensure that the medicines it is funding are genuinely benefitting patients and meeting the expectations patients have for their care, and as such is inherently attractive. Because OBP schemes rely on high-quality data on patients' treatment outcomes being collected and collated to determine a medicine's ultimate price, their use means putting in place the infrastructure and resources required to be able to collect and analyse this data. Adopting an OBP approach is a signal that the collection and use of data on the outcomes for patients is valued by the health system – and may encourage moves in this direction where this capability does not already exist.

A key element of this Phase 2 research project was to address how far the challenge of implementing OBP might practically be met, as well as what the benefits could be.

# 3 Research findings and discussion

## 3.1 Outcomes and data availability for candidate medicines

To identify what measures exist to capture medicines' impact on each of the four key outcomes for OBP (as derived from Phase 1)<sup>10</sup>, and whether any of these are currently collected within the NHS at scale, we undertook a *rapid assessment of the literature* and supplemented the findings with *expert interviews*. For details see Appendices 1 and 3.

### Key results

The most frequent measures identified were:

**Survival:** overall survival and disease-specific survival. For cancers with a relatively short median survival prognosis (e.g. less than one year), overall survival would be the preferred measure.

**Disease progression:** progression-free survival, relapse-free survival, objective response rate, or response evaluation criteria in solid tumours (RECIST). Alternatively, measures including time to progression, time to treatment failure, time to next treatment or treatment duration may be used as proxies.

**Long-term treatment side-effects:** as there is no national-level dataset that uniformly captures the broad variety of possible long-term treatment implications, the most appropriate measures of long-term side-effects need to be identified on a scheme-by-scheme basis.

**Return to normal activities:** the EQ-5D is the preferred measure currently available. It is also part of the Cancer Quality-of-Life Metric Project, in development by PHE (now NHS Digital) and NHS England, and is used in other (local) PROMS initiatives. Others include the EORTC-QLQ-C30 questionnaire.

### Survival

The most frequent survival measures identified in the literature review were *overall survival*, *all-cause mortality* and *disease-specific survival* (see section A in Appendix 1 for additional details on the search strategy and findings). However, definitions were not necessarily equivalent between documents or organisations. For instance, documents typically did not specify the start date used for survival (e.g. start of randomisation or treatment, or end of treatment), making it difficult to compare across sources.

As informed by our consultation with experts, the optimal measure of survival depends on a number of factors. For instance, survival can either be measured from start of treatment (probably the most appropriate starting point for drug interventions) or from the point of

histological diagnosis (expensive drug treatments are almost never used without histological diagnosis). Similarly, land mark time points can be used, but the relevant ones will vary by cancer site and type, for instance, one, two, or up to five years for lung cancer, but longer time points for breast cancer or thyroid cancer. Disease stage and prognosis were also identified as relevant factors to determine the optimal survival measure (see section C in Appendix 1 for further details.)

We propose that for cancers with a relatively short median survival prognosis (for example of less than one year), overall survival would be the preferred measure. For patients or populations with a longer survival prognosis, disease-specific survival would be the preferred measure as survival would more likely be affected by non-disease specific factors on which the medicine has no influence.

With respect to the time horizon for the assessment of survival, experts agreed on the convenience of measuring overall survival from the treatment start date, for a limited time-period that may vary by cancer type, line of therapy, or prognosis. For piloting the OBP scheme with a specific drug, the median overall survival as estimated in the main trial informing NICE's assessment of the medicine could be used to inform the most appropriate time horizon of the outcome measure. However, note that many of the newer drugs coming on the market may not have reached median overall survival in the trial, meaning these sources cannot always be used to estimate time horizons.

Regarding datasets available for tracking overall or disease-specific survival, a data feed from the Office of National Statistics (ONS) mortality registers as well as from the SACT data are included in the National Cancer Registration and Analysis Service (NCRAS). In addition, the assessment of survival in SACT uses the same time horizon as that suggested in the experts consultation.<sup>18</sup> Therefore, linking these comprehensive and reliable data sources means it is in principle possible to obtain the recommended measures of survival through NCRAS data.

## Disease progression, relapse and recurrence

The most frequent disease progression measures identified in the literature review (see section A in Appendix 1) were: *time to progression*, *time to treatment failure*, *time to next treatment*, *treatment duration*, *progression-free survival*, *relapse-free survival*, *objective response rate*, and *response evaluation criteria in solid tumours (RECIST)*.

While disease progression or relapse would ideally be collected prospectively as part of routine data collection in the NHS, there was consensus among experts that existing data sources are not sufficient for this, as (parts of) the information needed is captured in free text or non-standardised data fields. Proxies for progression or relapse, as suggested by the experts interviewed, are *time to next treatment* (when applicable), *treatment duration*, or *time to treatment failure*. These proxies can provide estimates which are relatively consistent with progression-free survival in some cases. Regarding *recurrence* most of the interviewed experts flagged that this is not consistently defined in routine practice. Hence, where a measure for recurrence is used as part of an OBP-scheme, this needs to be defined upfront.

Data collection to capture disease progression or recurrence is also challenging. No data fields related to this, or scan or test results to infer it from, are provided in the SACT database. Thus, at present, it does not seem possible to track progression-free survival through SACT. The SACT database does include the field 'Regimen outcome summary', which allows users to indicate whether the treatment regimen was stopped due to 'Progressive/recurrent cancer'. However,

this entry does not specify the date of progression or recurrence, potentially making it unsuitable for use in an OBP scheme with defined timepoints for assessing treatment ‘success’. The small percentage completeness of the non-mandatory field ‘Regimen outcome summary’ is an additional challenge: it was about 12% in 2017-18).<sup>19</sup> However, the percentage of completeness of mandatory fields is very large, and for cancer drugs on the CDF scheme, completeness of mandatory fields is often close to 100% (see for instance *Data Completeness Report: April 2018 – March 2019*).<sup>20</sup> Turning the field into mandatory and setting the right incentives and resources could be the answer to this challenge.

The experts who were interviewed (see section C in [Appendix 1](#)) observed that collecting data on *treatment duration* seemed more realistic, since it would be possible to use the same methodology as that for drugs funded by the CDF source of funding: using the earliest date out of ‘Start date of regimen’, ‘Start date of cycle’ and ‘Administration date’ as treatment start date, and the latest date of the same three items as the patient’s final treatment date.<sup>18</sup> The treatment duration is then calculated as the difference between these two dates, plus any administration interval (measured in days).<sup>18</sup> Note that *treatment duration* is one of the main analyses provided in CDF annual and final.<sup>18</sup> This way, treatment duration or another proxy can provide a way to capture disease progression in an OBP scheme through data already collected as part of SACT.

## Long-term treatment side-effects

The literature review showed that side-effects of treatments are highly varied depending on the location of disease and the treatment prescribed (see section A in [Appendix 1](#) for further details and definition). In general, we found that due to the wide range of side-effects relating to different disease areas and treatments, it is highly unlikely that they can be captured with a single instrument. The search did not provide any reliable measure to capture the overall impact of long-term treatment side-effects. In addition, there was again a general agreement among experts that a ‘one size fits all’ approach does not apply especially in relation to this outcome, as side-effects from cancer treatment vary by type and stage of cancer, treatment modality used, plus patient-to-patient variation (additional information can be found in section C [Appendix 1](#)). Therefore, the experts advised to identify on a scheme-by-scheme basis the most common long-term treatment side-effects (such as fatigue, pain, and difficulties with focused thinking, ideally linked to the use of the medicine) that are most important from the patient’s perspective.

We have not identified a national-level dataset that fully captures long-term treatment side-effects in a way that can be readily used for OBP<sup>vi</sup>. Yet, the research showed that in principle it should be possible to determine long-term treatment side-effects through databases such as the Clinical Practice Research Datalink (CPRD), which is linked with relevant, nation-wide datasets such as Hospital Episode Statistics (HES) and NCRAS. (Other available datasets are described in section B, [Appendix 1](#)). However, patients are not regularly screened for long-term side-effects, and therefore they would only appear in the data if the patient had visited their GP or the hospital for such a problem (for example, a patient’s thyroid function problems would

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<sup>vi</sup> We note that while SACT captures ‘toxicity’ in the list of reasons for discontinuing a treatment, which is an important short-term adverse event that may be included in an OBP when deemed relevant for a specific treatment, it does not include specific fields regarding long-term side-effects of treatment.

only be picked up when that information was entered in GP or hospital records).

Meanwhile, there is at least one example at the local level, where patient-reported outcome measures (PROMs) are captured systematically in a way that would make those data useful for an OBP-scheme. Interviews with experts (see Appendix 3) at The Christie showed that the *ePROMs Team* at that Trust is collecting data directly from patients through a survey which, amongst others, includes a list of the most common/important long-term side effects of treatment. Patients are contacted by the ePROMS team at The Christie during their first visit and prompted to respond to the questionnaires via text or email after every appointment during treatment, and during follow-up, which may be as long as five years. All PROMs are collected through the 'DrDoctor' data platform. Data can be linked with electronic health records at The Christie using the patient's NHS number as a unique identifier. The time between end of treatment and discharge is quite variable, from 4-6 weeks to 5 years. About 40% of patients completed the surveys in the last collection point (usually in the last appointment before discharge), which has the lowest completion rate across the times that each patient is prompted to complete the questionnaire. The research undertaken does not allow us to generalise the completion rate in other settings beyond The Christie.

Based on these results, it appears that at present bespoke data collection arrangements would need to be put in place as part of any national OBP scheme incorporating long-term side effects as an outcome, together with an agreement of a clear and consistent definition of the specific side effects to be captured and how these are to be measured.

## Return to normal activities

Although our literature review identified more than 40 different measures to capture the ability to perform normal activities of daily life, all the interviewed experts agreed that there are currently no national databases with structured data on ability to return to normal daily activities for patients treated for cancer (see Appendix 1). While an assessment of activities of daily living is performed during nurse and clinician consultations, the experts interviewed agreed that these are not usually performed using standardised tools, and not recorded in ways that can be easily extracted and analysed at scale. For the purpose of OBP schemes, interviewed experts preferred generic health-related quality-of-life (HRQoL) measures over condition-specific ones. The rationale provided was based on practical issues (including data availability) as well as additional concerns regarding limitations in comparability across OBP schemes if condition-specific measures were used. There was also concern regarding the general difficulty of defining what a "normal activity" means for patients at different cancer stages.

The recommendation of using generic quality-of-life measures to capture return to normal activities is supported by literature on PROMs: there is evidence that generic measures like the EQ-5D, the Short-Form 6 Dimensions (SF-6D), and the Health Utilities Index (HUI-3), all of which include at least one question related to usual activities as work, study, housework, family or leisure, perform well in capturing health-related quality-of-life for cancer patients, in terms of validity and responsiveness of the measure, for most cancers.<sup>21-23</sup> Also, the return to normal activities dimension of the EQ-5D is independent of the other four dimensions.<sup>24</sup> A review on psychometric properties of these measures at cancer site level showed that the EQ-5D is valid (in the sense that it can differentiate between severity groups of patients) and responsive (able



to detect appropriate change in quality of life over time points) instrument for cancer.<sup>25</sup> There was also evidence to support the validity and responsiveness of the HUI-3 (though more limited, compared to the EQ-5D), and little evidence to allow a judgement on validity or responsiveness of SF-6D in cancer.

The EQ-5D may be a good choice as the preferred HRQoL measure for an OBP scheme. It is the most widely used HRQoL measure globally and extensively tested in various settings.<sup>26</sup> It is also the most commonly used measure in England, since it plays a key role in NICE health technology appraisals and is also collected by some primary care centres.<sup>26</sup> While it is not routinely collected at scale either in the SACT dataset or any other national level dataset yet, PHE and NHSE&I are working on the Cancer Quality-of-Life Metric Project, which aims to collect data on the quality of life of all cancer patients who are alive 18 months post diagnosis, using the EQ-5D-5L and EORTC QLQ-C30 instruments. From a consultation with PHE, we learnt that the project has completed the pilot phase,<sup>27</sup> and that PHE and NHSE&I launched the survey in late 2020. It was expected that from September to November 2020 the project would collect data from a 10% sample of people diagnosed with breast, lung, and colorectal cancers, at the time of 18 months from their diagnosis.

Note that the Cancer Quality-of-Life Metric Project only collects PROMs at 18 months from a patient's diagnosis. (The implementation pilot study also collected information at 6 months and 12 months from diagnosis.) This is because the aim is to measure long-term QoL (as opposed to side effects of treatment), and 18 months was considered sufficient to cover most cancer treatments. Therefore, it will be essential that the Cancer Quality-of-Life Metric Project is flexible and extends the data collection to alternative times points (for instance, a 6 months follow-up for types of cancer with shorter survival), to make the dataset usable for an OBP scheme. Finally, note that the EQ-5D-5L is also collected by the ePROMs Team at The Christie,<sup>28</sup> which is an important facilitator for a potential pilot OBP scheme in Greater Manchester.

## Learnings and limitations

Our findings suggest that it is not currently possible to undertake the routine, at scale data collection required for an OBP scheme incorporating all four outcomes as set out in our Phase 1 report. Detailed results reported Table 8, Appendix 1. However, ongoing developments and further data collection initiatives would likely create the conditions that are necessary for such an OBP scheme to be agreed and conducted with success in the future. To illustrate, NCRAS (through the SACT dataset) already collects outcome data relating to (or serving as a possible proxy for) 'Survival', 'Disease progression, relapse and recurrence', and these data could be linked with external datasets that are more suitable to collect outcomes data relating to 'Long-term treatment side-effects' and 'Return to normal activities', such as the Cancer QoL Metric Project. The time frame for collecting data to determine whether treatment is successful is important to define and should be considered on a treatment-by-treatment basis. It should be noted that the longer the timeframe for data collection, the higher the uncertainty may be as to whether the outcome can be directly linked to the medicine, particularly for more subjective outcome measures.

In the short-run, an OBP scheme based solely on outcomes of a more clinical nature may also be feasible, though this would not capture the quality of life outcomes and hence may fail to reflect the drug's full value to patients.

Table 1 summarises which outcome measures and data sources would be the most appropriate

for a realistic and pragmatic OBP scheme. A detailed illustration of a hypothetical OBP scheme is provided in Appendix 1, to illustrate the feasibility of such a scheme while noting the assumptions made.

Table 1: Overview of core outcome measures

<b>Survival</b>		<b>Disease progression, relapse or recurrence</b>	
<b>Survival prognosis</b>	Suggested outcome measures	<b>Line of treatment</b>	
<1y	1. All-cause mortality 2. Disease-specific mortality	1, 2	3 +
>1y	1. Disease-specific mortality 2. All-cause mortality	>1y	1. Progression-free survival 2. Relapse-free survival 3. Time in remission 4. Time to next treatment 5. Treatment duration 6. Time to treatment failure
		<b>Prognosis (survival)</b>	1. Relapse-free survival 2. Progression-free survival 3. Time in remission 4. Treatment duration 5. Time to treatment failure 6. Time to next treatment
		<1y	1. Time to next treatment 2. Treatment duration 3. Time to treatment failure 4. Progression-free survival 5. Relapse-free survival 6. Time in remission
			1. Treatment duration 2. Time to treatment failure 3. Time to next treatment 4. Relapse-free survival 5. Progression-free survival 6. Time in remission
<b>Long-term treatment side-effects</b>		<b>Return to normal activities of daily living</b>	
<b>Long-term treatment side-effects</b>		<b>Return to normal activities of daily living</b>	
Common symptoms include:	Other specific symptoms vary by:	EQ-5D-5L questionnaire:	
- Fatigue - Diarrhoea - Nausea - Pain - Cognitive impairment	- Cancer site/type - Disease stage - Treatment modality	Dimension "USUAL ACTIVITIES (e.g. work, study, housework, family or leisure activities)"	
		[1] I have no problems doing my usual activities [2] I have slight problems doing my usual activities [3] I have moderate problems doing my usual activities [4] I have severe problems doing my usual activities [5] I am unable to do my usual activities	
		EORTC-QLQ-C30 questionnaire:	
		Modules related to usual activities during the past week:	
		- "Were you limited in doing either your work or other daily activities?" - "Were you limited in pursuing your hobbies or other leisure time activities?"	

The main limitation is that data availability for core outcomes of an OBP should not be considered static. Collection of clinical outcomes is relatively advanced and could serve as a starting point for OBP schemes, while data linkages between these and the other core outcomes can be established through pilots and at scale as data collection initiatives are evolving for 'Long-term treatment side-effects' and 'Return to normal activities'.

## 3.2 Proposed OBP scheme

Following preparatory briefing by the research team, who drew on the findings of the literature review and expert interviews in combination with the outputs from Phase 1 of the project,<sup>1</sup> a consensus workshop was held with the Steering Group in February 2020. The workshop was to agree the characteristics of a theoretical OBP scheme to be tested in interviews with NHS hospital staff, managers at national NHS stakeholders and in industry (reported in section 3.3 below) and in a simulation modelling exercise reported in section 3.4) and as the default starting assumption for Phase 3 of the OBP project.

At the workshop the members of the Steering Group were asked for their views on the type of OBP scheme that would be most likely to be used in practice. After discussion in three separate break-out groups and then in plenary, the Steering Group members came to a consensus about some of the characteristics of the OBP scheme for the rest of the Phase 2 project and the default starting position for a later Phase 3 project. A further characteristic of the proposed default OBP scheme was whether treatment success should be determined on the basis of a composite weighting of the four types of outcomes, or by a threshold level of each outcome being achieved; this was discussed via email correspondence between the research team and the Steering Group and was confirmed at the following meeting of the Steering Group.

A second workshop discussion was held with the Steering Group in June 2020, again following preparatory briefing by the research team, to agree the principles used to define ‘successful’ treatment in the context of such a scheme. These principles are also set out in the rest of this section of the report. The Steering Group members who participated in the two workshops are listed in Appendix 2. The key results from the workshops and correspondence with the Steering Group are summarised in the following box.

### **Key results**

A default set of characteristics for future OBP schemes could specify:

- Prices and rebates are linked to treatment outcomes for individual patients, rather than average outcomes across populations
- A two-level price, with an initial payment by the NHS and a subsequent rebate to a pre-agreed lower price level if the pre-agreed, expected ‘success’ levels of patient outcomes are not met
- ‘Successful’ treatment is defined as where patient outcomes meet all of the separate threshold levels set for each of the individual outcome measures
- Outcome ‘thresholds’ indicative of treatment success can be based on available clinical trial data and available real-world-data for an outcome metric, but should also align with points identified in NICE’s HTA process that determine cost and clinical effectiveness
- The scheme should use all four outcomes types identified in Phase 1, with omissions agreed between all parties on a case by case basis. Not already having NHS data readily available should not on its own justify omitting an outcome type

More complex forms of OBP are conceivable in the longer-term, but the characteristics listed above represent a pragmatic first step.

## Scheme characteristics

As a result of the Steering Group consensus workshop in February 2020, and of the follow-up email correspondence between the research team and the members of the Steering Group during March and April 2020, the OBP scheme to take forward for the rest of the Phase 2 project was agreed to have the following four characteristics, for the reasons given below. A scheme with these characteristics is recommended as the default for piloting an OBP scheme in a future Phase 3 project. Pragmatically, it is desirable now to pilot the necessary data collection for multiple outcomes and using those data in practice to determine how much the NHS pays for a cancer medicine. More complex forms of OBP could be negotiated in the future if the NHS and manufacturers see advantages to patients and themselves in doing so.

### **Individual patient outcomes rather than average population outcomes are the preferred basis for an OBP scheme**

The OBP scheme that the research team referred to in interviews with NHS staff and other stakeholders (see section 3.3) and used in the retrospective analysis (section 3.4) is based on the treatment outcomes recorded for individual patients, rather than average outcomes across a population of numerous patients over a period of time (e.g. each year). Thus, if a patient's treatment yields good outcomes, then the payment for that patient's medicine would be higher than if that patient's treatment yields poor outcomes.

The rationale for favouring an individual patient basis is that if a rebate is due to the NHS because a treatment has not been successful it can be paid to the NHS as soon as the unsuccessful outcome is known. Allowing for the lag in collecting outcomes data, determining the payment made on a patient by patient basis avoids the need to wait even longer while the outcomes for a whole population over a year (or other period of time) are collected and averaged. Both approaches require the same individual outcomes data to be collected. Nevertheless, the Steering Group concluded that a population approach should not be ruled out entirely and could be reconsidered in future.

### **A binary payment scheme was favoured with one price for the medicine when treatment is successful and a lower price when it is not**

During the Phase 1 project, it had already been determined that an OBP scheme that made the payment by the NHS to the manufacturer of the medicine a continuous function of a (composite) measure of outcome would be too complex to negotiate and administer. Further discussion with the Steering Group at the February 2020 workshop during the current Phase 2 research, went further and determined that the preferred basis for OBP would be for one level of payment if the treatment is successful for an individual patient and a lower, even zero, payment if the treatment is unsuccessful for that patient. Thus, for any medicine there would be only two possible levels of payment, not more (e.g. no third, intermediate, level of payment for merely moderately successful treatment).

Binary OBP was favoured at this early stage in developing such schemes, because of the need for simplicity at the outset. The possibility of moving eventually to OBP schemes with more than two levels of payment (i.e. stepped schemes, and/or increased payments if the medicine exceeds the agreed success threshold for each outcome) should be kept open in the longer term as they would permit a closer relationship between what the NHS pays for a medicine and the outcomes that the medicine achieves for patients.

## **The manufacturer should pay the NHS a rebate if treatment with the medicine is unsuccessful**

On grounds of practicality, the initial price paid for the medicine will likely be that which corresponds to an assumption that treatment is successful, with a subsequent rebate if outcomes turn out to be poor. (As opposed to the initial price being that which corresponds to an assumption that the treatment would be unsuccessful, followed by an additional payment if outcomes turned out to be good.)

It is unlikely that manufacturers would agree to make a medicine routinely available to the NHS with zero or low payment, and as the main purpose of OBP is to facilitate earlier patient access to effective new medicines, it would be counterproductive to complicate and extend the negotiation of the scheme. However, if stepped schemes are considered again at some future point (discussed above) it may be preferable for the initial price to be set at an intermediate level within such schemes, so that financial risk is shared between the payer and the provider. In that way, the price could go up or down or remain at the initial level, depending on the outcomes achieved.

## **Identify a separate threshold for each core outcome to determine the success or not of treatment with the medicine**

Following the February 2020 workshop, the research team sought the Steering Group's views in an exchange of emails on options for accommodating multiple outcome measures in OBP schemes. A novel feature of the OBP arrangement proposed on the basis of the Phase 1 research, compared to existing OBP schemes internationally, is relating payment to multiple outcomes rather than just one. Having multiple outcomes necessitates decisions about how to take them all into account when determining the payment due for a medicine. The options are to define successful treatment in either of the following ways:

- A. When the level of one **composite** outcome measure for each patient that weights together all of the chosen outcome metrics passes a threshold level; or
- B. When threshold levels for all of the individual outcome measures **separately** are passed. Failure to meet the threshold level for any one of the outcome metrics is taken to indicate unsuccessful treatment. (Thresholds would be set as part of negotiation between the manufacturer and NHSE&I based on the individual medicine in question and taking into account what would be achieved by current standard treatment in the absence of the new medicine.)

The research team proposed to the Steering Group that the OBP scheme for the purposes of this project should be of type B: separate thresholds for each outcome metric. This option was accepted by the Steering Group as a pragmatic way forward and to be the focus for the rest of the Phase 2 project and the default for any future Phase 3 piloting of an OBP scheme. Thus, rather than a composite outcome measure, there would be success threshold levels set for all of the individual outcome measures separately. Failure to meet the threshold level for any one of the outcome metrics would be taken to indicate unsuccessful treatment<sup>vii</sup>. The reasons for favouring the separate thresholds approach are as follows.

For either type of OBP scheme it is necessary to collect the same information about each patient receiving the medicine, namely the levels of their outcome metrics for all outcomes included in the scheme. Similarly, for either type of OBP scheme it is necessary to agree in the

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<sup>vii</sup> The simulation analysis reported in Section 3.4 assesses the impact of relaxing the requirement to meet all four thresholds if data are lacking for one or more of the outcomes.

negotiation between the pharmaceutical company and the NHS negotiator what level of each outcome metric is consistent with the medicine being deemed a successful treatment. No further negotiation is then required for a type B OBP scheme.

However, for a type A OBP scheme it would be necessary to then negotiate and agree the weights to be attached to each outcome metric in order to combine them into a single composite metric. None of the existing OBP schemes found in the literature review for the Phase 1 project relies on a composite outcome measure to determine treatment success. It seems that negotiation of OBP schemes hitherto has focused on the one outcome in each case about which there remained the greatest uncertainty.

It is also the case that a great deal of study has gone into, and debate has surrounded, the determination of the weights attached to the constituent elements of composite outcome measures hitherto, not least those that are used to estimate the quality-adjusted life year (QALY) metric favoured by NICE. It seems likely, therefore, that including such a weighting process as part of a price negotiation would be burdensome and unpopular with price negotiators in both industry and the NHS.

Consequently, the default approach to OBP is assumed to be on the basis of a separate success threshold being set for each outcome metric. A more complicated OBP scheme based on a composite indicator formed by weighting together a number of metrics remains a possibility to reconsider in future.

## Considerations for agreeing success thresholds

Given an OBP scheme with the above characteristics, the members of the Steering Group were asked in their June 2020 online meeting to consider:

- 1) what are the main considerations that need to be resolved in agreeing clinical 'success' thresholds for each of the four types of core outcomes (survival; disease progression; long-term treatment side-effects; return to normal activities) for inclusion in an OBP scheme; and
- 2) for which of those is it possible to propose general guidance, agreed by NHS/payer, clinical, patient and industry representatives in advance of any individual negotiations between the NHS and the manufacturers concerning specific medicines?

The relevant considerations – proposed by the research team and refined by the Steering Group – for agreeing success threshold levels for outcome metrics for an OBP scheme are:

- Whether to omit any of the core outcomes for an OBP scheme should be decided on a medicine-by-medicine basis;
- Outcomes to be included would not need to be limited to those measured in clinical trials of the medicine;
- Interaction with NICE processes can be on the basis of OBP as an option from the outset;
- What to assume when data are missing;
- Who should oversee the design and governance of any new data collection;
- Who should oversee the outcomes data collation and analysis.

These considerations are discussed in turn in the following paragraphs.

## **Whether to omit any of the core outcomes for an OBP scheme should be decided on a medicine-by-medicine basis**

The *a priori* grounds for omitting an outcome type could be that:

- The outcome is irrelevant to the medicine – e.g., ‘return to normal activities’ where that is not likely to be possible for many indicated patients.
- No great decision uncertainty exists that is driven by the medicine’s effect on that outcome.
- Where including an outcome type might create perverse incentives that could be harmful to patients, e.g., encouraging clinicians to delay scanning to see if a tumour has progressed.

Other than in the above cases, which outcome types to include should be for negotiation between the NHS and the manufacturer (with input from relevant stakeholders as appropriate, e.g. patient representatives) on a medicine by medicine basis, and based on the evidence available and its assessment (for example, by NICE). The starting point should be to use all four core outcomes types, as these were found to be important to patients<sup>10</sup>.

Ideally, a decision to omit any of the four core outcomes should be agreed upon by all parties involved. Not already having the real-world data readily available on an outcome type should only be a lower-ranking consideration in prospective OBP-schemes, as it may be worthwhile initiating collection of data on important treatment outcomes, where those data do not currently exist. While patient reported outcomes, including how far patients have been able to return to normal daily activities, are likely the most challenging outcomes to incorporate in an OBP scheme, these are also the outcomes types that would allow OBP to better reflect the benefits of the medicine to the population treated.

### **Outcomes to be included need not to be limited to those measured in clinical trials**

Allowing outcomes that are important to patients to be part of an OBP scheme is desirable, whether or not those outcomes were measured during clinical trials of the medicine, to ensure a full assessment of the benefits and harms of a medicine. OBP may prove to be an effective way to incentivise routine data collection on those important outcomes. If no clinical trial data are available for a particular outcome metric, the success threshold may be benchmarked against the ‘success’ of current care, as could be derived from published studies (and complemented with the elicited expert opinion where needed).

To the extent that clinical trial data for an outcome metric are available and are being presented as the outcome level expected from a new treatment when in routine use, this would constitute an acceptable threshold for ‘success’ to be measured against.

### **Interaction with NICE processes can be on the basis of OBP as an option from the outset**

The main advantage of having the option of an OBP scheme is, as described earlier, where this permits earlier patient access to potentially effective new medicines. Thus OBP needs to be considered as an option from the outset, particularly when decision uncertainty about cost-effectiveness is likely to be driven by uncertainty regarding real world usage and effectiveness (not price) of the medicine, and where a simple discount thus would not be an appropriate solution. As described earlier, in Chapter 2, OBP can be an alternative to the CDF, i.e. as the outcome of a NICE appraisal where the uncertain evidence base could not support a recommendation for baseline commissioning. However, manufacturers could also be permitted the option to propose an OBP scheme from the outset, with NICE’s assessment being

on the basis of the proposed OBP scheme in place of a simple price. Thus, the OBP scheme would not bypass NICE's HTA process but rather complement it, by offering more flexibility for manufacturers to propose and/or NICE to recommend cost-effective access to beneficial new treatments.

#### **What to assume when data are missing**

What is assumed about the success of cases where data are missing will, with rational negotiators, be reflected in the initial price agreed with the payer as part of the OBP-scheme. For example, if treatment is assumed by default to have succeeded for a patient for which outcomes data are missing then the initial (default) price negotiated by the payer can be lower than if treatment is assumed not to have succeeded in such cases. Thus, what to assume when outcomes data are missing can be decided upon as part of the initial NHS/manufacture negotiation of the OBP scheme.

#### **Who should oversee the design and governance of any new data collection**

If OBP is to become an established option for paying for new medicines, then it would make sense for the process for collecting and collating outcomes data, and the governance of that process, to be determined in advance so that the same approach can be adopted by all OBP schemes, not left to medicine-by-medicine negotiation. This might mean that the industry (perhaps coordinated via the ABPI) should negotiate with the DHSC or NHSE&I, with appropriate input from patient representatives, whose data these are. One solution could be for NHS Digital (previously PHE) to oversee the design and governance of data collection, given their equivalent role in curating data collection agreements in the CDF, and given the important role that their SACT dataset plays in capturing treatment outcomes, which are likely to include health-related quality of life measures in the near future.

#### **Who should oversee the outcomes data collation and analysis**

A trusted third party (independent of the NHS and the manufacturer) should be responsible for the collection and analysis of data that help determine whether a rebate is due, a decision which is then to be agreed between clinicians, manufacturers and the NHS. Various firms have developed software specifically for this purpose and some already work with the NHS to provide real time data insights about the number of patients enrolled in an OBP, their status compared to the success thresholds over time, etc. An organisation such as NHS Digital (previously PHE) could act as a trusted third party. This approach is consistent with what NHS Digital does for the CDF currently with respect to data collection.

## **3.3 Practical considerations for implementing OBP in the NHS**

The practicalities of collecting and collating outcomes data and using these to determine outcome-based payments, were investigated via interviews with operational staff in various NHS Trusts delivering cancer services, with key staff in the NHS, NICE and PHE, and with pharmaceutical industry managers. To understand patient experiences of data collection and their views on the potential burden on reporting outcomes data required for an OBP scheme, a focus group discussion with patients was conducted. Details of the methods for the interviews and the focus group are reported in Appendices 3 and 4 respectively.



## **Key results**

To support outcomes data collection and ensure it is efficient, standardised approaches could be established. For patient-reported outcomes, this could be through the creation of a patient portal. In addition, standardised templates and reminders could be set up for clinicians to report data.

OBP imposes extra demands on hospitals where these schemes are in operation. These include:

- financial costs, e.g. for additional data storage or setting up patient-reported outcome platforms
- staff time collecting additional data on patient outcomes.

Processing requests for rebates will impose a burden some individual NHS Trusts may not have capacity to meet, so this might better be coordinated centrally by NHS England. Clarity is also needed on whether any rebates would remain with NHS England or would be passed back to the Trusts treating patients with the medicine.

Given the practical challenges involved, OBP cannot be implemented without buy-in from groups including clinical staff, Trust executives and national system stakeholders. Ensuring these groups are aware of why the underpinning data should be collected and the benefit to patients is essential.

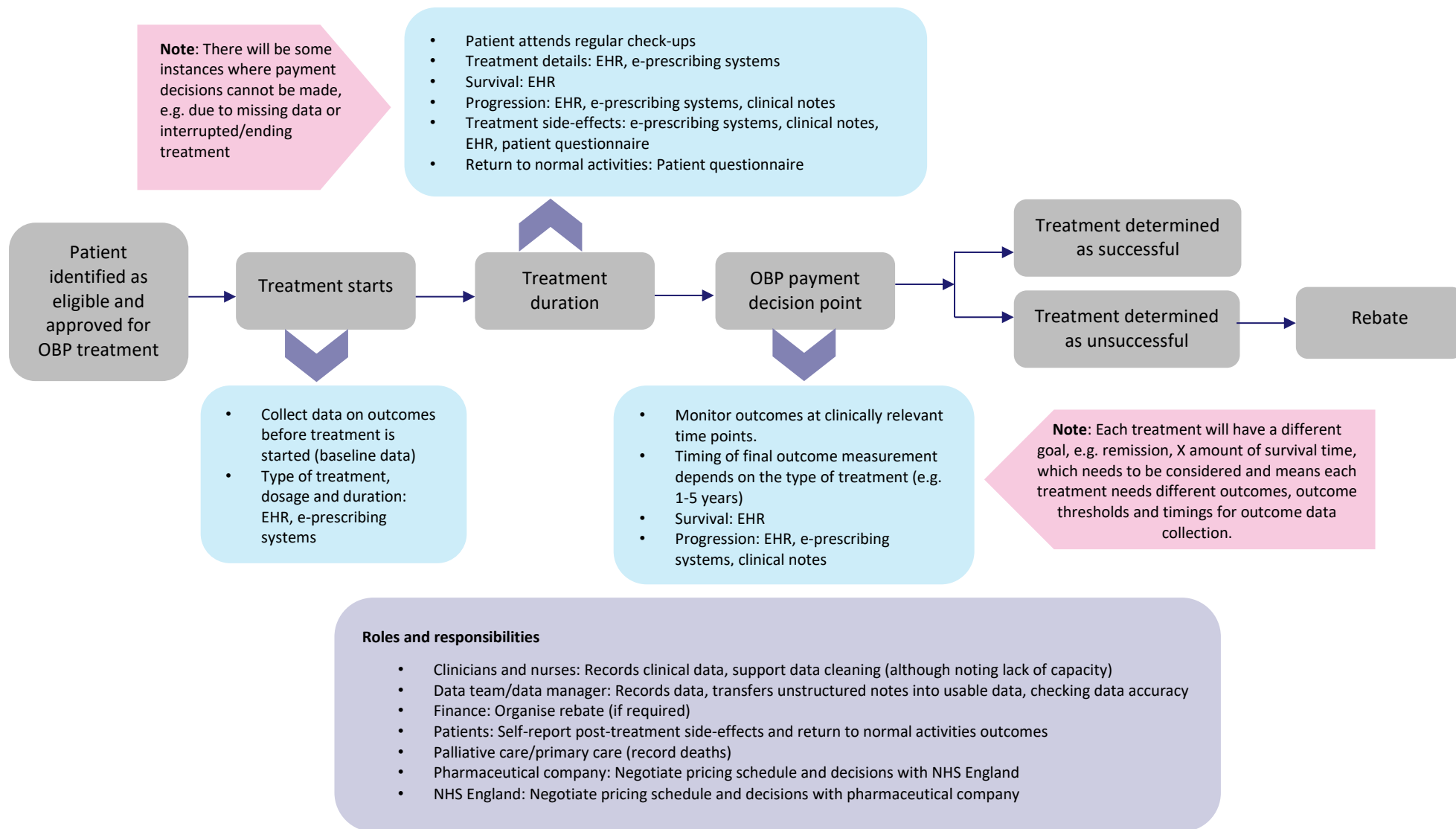
Buy-in from patients is also fundamental. In our focus group, patients were supportive of an OBP approach on the condition they would know, and have control over, exactly how and why their data are being used.

Given the need for an OBP scheme to access, track, and link individual patient data across multiple datasets, a clear and robust framework for information governance is required. Patient consent for their data to be used in an OBP scheme should be obtainable provided the appropriate policies, procedures and safeguards are put in place.

## **Outline of what an OBP scheme might look like**

The interview data covering the current data collection, what additional data needs to be collected for OBP and other requirements for an OBP scheme were analysed by the research team and a flow diagram developed outlining the different OBP stages and the requirements for each (as well as staff roles and additional aspects to consider). This diagram is presented in Figure 3. The following sections discuss key areas of what needs to be considered or improved to be able to implement an OBP scheme:

- expanded mechanisms are needed to collect patient outcome data in a usable format for OBP;
- the potential time and cost burden of OBP needs to be recognised;
- details of the rebate process need to be clarified;
- the need to achieve buy-in from NHS staff and organisations;
- the need for patients to buy-in to their data being used for OBP;
- a clear and robust framework for information governance is required.



Key: Grey box = Key points in the OBP pathway; Blue box = What types of data need to be collected and how; Pink box = Points to consider for data collection; Purple box = Roles and responsibilities

Figure 3 Flow diagram of an OBP scheme and the requirements at each stage

## Expanded mechanisms are needed to collect patient outcome data in a usable format for OBP

The interview results confirm the findings reported in Section 3.1, by showing that some key types of cancer outcome data are already collected (notably treatment information, survival and, to some degree, disease progression) in a structured format. The quality of life data needed to measure long-term treatment side-effects and a patient's ability to return to normal daily activities are collected as well (e.g. in nurses or clinician notes), though rarely in a formal, systematic way as would be required for usage in an OBP. In addition, some hospitals are still transitioning from paper-based to electronic data collection which means the outcome data that are already collected are in clinical notes, which are difficult to extract from and analyse.

Data that are collected in a format most appropriate for use in an OBP scheme are in electronic health records (EHRs) and e-prescribing systems, although it was noted that secondary/tertiary care e-prescribing systems do not always have high completion rate by clinicians. This is in part due to a lack of incentives and motivations for already busy clinical staff to spend additional time recording patient data, as illustrated by the following quote from an interview with a non-clinical NHS operational staff member at Essex Partnership University NHS Foundation Trust.

*"It [time spent collecting outcomes data] varies massively. The handful of people we've got doing it are so valuable to the clinicians they work with because they recognise how much time they would have to spend otherwise. On the whole most of our data come from clinicians themselves either once a month or a couple of times a year. They spend a significant time updating records and reviewing that information. Equally, some teams don't get it done."* (Other operational role, Essex)

It is important that mechanisms are put in place to be able to collect usable patient data on all four outcomes of interest for this proposed OBP scheme. Participants outlined ways in which this can be achieved which includes using existing data collection methods and suggestions for additional forms of data collection. We understand, in particular, that there are up to 100% data completeness rates for SACT fields for drugs in the CDF, and that PHE (now NHS Digital) dedicates resources (mainly in the form of staff time) to achieve this.

**Survival and disease progression/relapse/recurrence data** can be collected and monitored using EHR and e-prescribing systems. While methods to extract these data from written clinical notes could be developed, it would likely be easier and more efficient to input data electronically from the outset (e.g. into EHRs). Details on survival need to be provided from ONS mortality data when death occurs outside hospital.

**Long-term treatment side-effects cover both clinical and patient-reported data.** Therefore, these data could be collected using EHR data (or clinical notes if necessary) and in self-reported forms by patients, such as through a validated questionnaire. Return to normal activities could also be measured through these self-reported patient questionnaires. It was suggested that to improve completion rates, efficiency and ease for patients to provide the information, that these data could be completed online, such as via a patient portal. Patients could then provide these data in their own time, although alternative arrangements would need to be made for patients without access to the internet.

*"The system that is missing is the one to record patient outcome data... There are bespoke systems in a number of areas around the country, but what platform is the NHS going to use to work out what value patients get from treatment?... That system might cost the trust hundreds of thousands of pounds. If the NHS thinks there is value in*

*working out what benefit patients are getting, NHS UK should be funding that system and making it is a resource that is available. Getting patient input is incredibly important, but the reason we don't do that at the moment is that there isn't a system to collect that data.”* (Cancer clinician, Leeds)

To support data collection and ensure it is efficient, standardised approaches could be established. As mentioned previously, for patient-reported outcomes, this could be through the creation of a patient portal which would collect the same quality of life data from all patients. For example, at The Christie NHS Trust, PROMs are collected directly from patients through the ‘DrDoctor’ data platform. In addition, standardised templates and reminders could be set up for clinicians to report data. Going further, some or all of the data fields could be made mandatory for clinical staff to complete to reduce the amount of missing data, as suggested by one of the interviewees:

*“I think you could do it so you had compulsory fields that had to be filled in so that you got a warning when it needed to be filled in. So for example, say you set up a certain drug, and you needed to fill in an outcome three months from the date of the patient's last treatment cycle, so something then would flash up to say you need to put an outcome in. Then you could forward that to the MDT [multi-disciplinary team] coordinator to fill it in and that would trigger the payment. At the moment, there is no reminder on there.”* (Pharmacist, Manchester Royal Infirmary)

In addition to collecting the necessary data in a format that can be used for OBP, the ability to clean the data and link these datasets to enable financial decisions (whether a rebate is due) needs to be considered. In terms of cleaning the data, this has the potential to require a lot of resources to reach the quality needed to make financial decisions, as has been seen in the investment needed to collect and prepare data for PHE for the CDF.

While data liaison teams could be used to support the data collection, it was noted that clinical staff will need a role in this to maintain clinical accuracy. In relation to data linkage, interviewees found this more difficult to comment on, as it was beyond their direct experience, although some noted ongoing initiatives to link datasets. Data linkage is made more complex by the different data collection systems used across and within hospitals.

In summary, to support outcomes data collection and ensure it is efficient, standardised approaches could be established. For patient-reported outcomes, this could be through the creation of a patient portal. In addition, standardised templates and reminders could be set up for clinicians to report data.

## **The potential time and cost burden of OBP needs to be recognised**

The potential burden of an OBP scheme was explored within the interviews and focus group and these largely covered the themes of staffing, cost and time pressures. As this focuses on the *potential or expected*, rather than an actual burden already being experienced, participants found it challenging to estimate the scale of these burdens and the amount that would need to be invested to minimise these.

The interview results indicated it is important that dedicated staff are put in place to implement and oversee OBP within a hospital. This could be created by expanding existing staff roles to incorporate tasks for OBP, or additional staff could be hired (or a mixture of these two options). The approach selected by hospitals would depend not only on the number of medicines and

patients in OBP schemes, but also on the staff they have employed already (e.g. more clinical coders or similar data management roles).

These staff would be needed to deal with additional data collection, cleaning and analysis/linkage to make financial decisions. Data teams/managers may be the most effective role to run an OBP scheme due to time pressures already faced by clinical staff. These data teams could be involved in recording data, implementing technical solutions to replace hand-written clinical notes with an electronic form and checking data accuracy, for example. However, clinical staff will likely need to be involved to some extent to support in the collection of clinical data and cleaning data.

There are financial costs that will be associated with implementing and running an OBP scheme. These financial costs are likely to be primarily data-related, such as additional data collection and cleaning and costs associated with linking datasets and data storage space. Other costs may also occur, such as if additional staff need to be hired.

While it is difficult to estimate these costs based on the high-level OBP outline proposed in this report, a PHE member of the Steering Group provided a list of 15<sup>viii</sup> factors identified by NHS Digital that can be assessed to estimate costs, including aspects such as training and guidance, transcription (manually re-recording data in a different format to which it was gathered) and transmission of data to the requestor.

*“Without a doubt there would be additional costs and some of those could be significant. Some of them would be perhaps developing into areas that we traditionally haven't captured data, like when we talk about patient reported outcomes and wellbeing and some of the social aspects of some of the metrics. Those would be quite difficult to collect and we'd have to develop online systems for patients to report those in and for us to follow-up with patients who weren't compliant with the reporting or who needed support... I would guess there would be a considerable cost, which would have to be balanced out against...savings.”* (Pharmacist, Manchester Royal Infirmary)

There is the potential for time pressures associated with running an OBP scheme, particularly for clinical staff. Although collecting data on the outcomes achieved for patients is intrinsically important, additional tasks associated with data collection and cleaning for OBP could be seen as an unwelcome additional burden for clinical staff who already lack time. As has been mentioned, dedicated data teams/managers could be hired to oversee OBP tasks which would minimise this burden on clinical staff, however clinical staff would still need to provide some input (such as collecting clinical data and supporting cleaning).

To the extent that OBP schemes could benefit medicines manufacturers as well as patients and the NHS, it would be appropriate for industry to share with the NHS the burden of the costs identified here. The extent and mechanism for such cost sharing would have to be a part of the negotiation of any individual OBP scheme between NHSE&I and the medicine manufacturer.

In summary, OBP imposes extra demands on hospitals where these schemes are in operation. These include:

- financial costs, e.g. for additional data storage or setting up patient-reported outcome

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<sup>viii</sup> The 15 factors are: System change and development; design, scope, general management and administration; training and guidance; gathering; data quality and validation; extraction; transcription; transformation; transmission; storage; analysis and interpretation; publication; consumption; destruction of data; and decommissioning.

- platforms
- staff time collecting additional data on patient outcomes.

## The details of the rebate process need to be clarified

In the proposed type of OBP scheme, the medicine manufacturer would pay a rebate to the NHS for every patient for whom pre-agreed levels of outcomes had not been achieved. Interviewees noted two potential challenges with the rebate process outlined for our proposed OBP scheme: the time inputs required to request rebates and uncertainty about who receives the rebate.

Firstly, a number of interviewees noted that hospitals may lack the capacity needed to process rebates. This was noted by an industry interviewee who had experience of working on an OBP scheme, stating that hospitals did not complete the required forms when they were entitled to a rebate and so did not receive the money:

*"[Our] OBP scheme, available on the NHS in England, ... came out in 2007 ... The hospitals found it challenging to apply for a rebate... We thought this would be ok because the outcome was based on something routinely collected and clear thresholds were set in terms of the reduction of protein for what counted as a success. [Company name] viewed this as a good scheme... but the trusts didn't return the form to get the rebate. They wanted to and saw value in the scheme, but didn't have time to return the form, so they didn't get paid [the rebate]. We had to follow accounting rules for pharma and the NHS, so we couldn't transfer the payments without the form completed. I don't think the scheme should be between pharma and individual hospital. It needs to be national through one financial body. This might be through NHS England or someone else. There needs to be an easier way to do this... It's not fair to put that admin burden on the NHS and frontline. It needs to be transacted centrally with a dedicated resource to complete the transactions."* (Industry)

However, we were unable to obtain an interview with a finance/procurement staff member to confirm these views. To help overcome the difficulties that individual NHS Trusts might face, it may be preferable, as this interviewee proposed, for the rebate process to be run centrally through NHSE&I, who would have negotiated the OBP with the medicine manufacturer.

Secondly, there was uncertainty among interviewees about who would receive the rebate. To give an incentive to collect the outcome data needed for OBP, it would be helpful if the NHS Trust whose patient's treatment turned out to be unsuccessful were to receive the rebate. Thus, there would need to be an arrangement for NHSE&I to pass the rebates back to the NHS Trust.

In summary, processing requests for rebates will impose a burden some individual NHS Trusts may not have capacity to meet, so this might better be coordinated centrally by NHSE&I. Clarity is also needed on whether any rebates would remain with NHSE&I or would be passed back to the Trusts treating patients with the medicine.

## Need to achieve buy-in from NHS staff and organisations

There is a need to create buy-in and incentives for OBP, both for clinical staff and national stakeholders (including NHSE&I).

There are currently few incentives for clinical staff to collect data they do not use for individual patient management and decision making. This contributes to the collection of poor-quality data and to missing data. However, it might disappoint patients that data on outcomes they

rate as highly as the extent and severity of post-treatment side effects,<sup>1</sup> and their ability to return to normal activities of daily living, are ultimately not considered in routine collection and monitoring due to staff capacity or data quality constraints. Motivation and incentives could be reinforced by informing clinical staff of why the data is being collected and what the benefit is to their patients.

As well as informing clinical staff of the value of an OBP scheme, the same could be done to develop buy-in from national system stakeholders, such as NHSE&I and trust executives. For example, the benefit to patients (through potentially quicker treatment access) and the financial benefits (i.e. the NHS receiving money back for ineffective treatments) needs to be made clear.

The effectiveness of actions to encourage buy-in is something that could be tested in further research in a Phase 3 pilot of an OBP scheme. We say more about the scope of such piloting later in the present report.

In addition, patient-reported outcome measures (which could be used to collect data on long-term treatment side-effects and return to normal daily activities) can be seen as subjective. Therefore, the measures used to collect self-reported patient data need to be robust and standardised, and this needs to be made clear to national stakeholders.

Overall, given the practical challenges involved, OBP cannot be implemented without buy-in from groups including clinical staff, Trust executives and national system stakeholders. Ensuring these groups are aware of why the underpinning data should be collected and the benefit to patients is essential.

## Need for patients to buy-in to their data being used for OBP

Focus group participants were generally positive about the use of an OBP scheme and they felt that if patients knew that the data were being collected to help the NHS obtain value for money, and to support other patients accessing effective treatments quicker, then patients would be happy to take part in additional data collection.

*“If you say to people we are asking you these questions because we want to know how effective the drug is because we pay for it on the basis of whether it works or not, then most people would think that’s a good idea because we want to get value for money.”*  
(patient focus group)

Quality of life discussions were said to be commonplace in consultations, with all the participants reporting that their quality of life was asked about in some respect during their consultations (e.g. consultant asking how a patient is feeling, asking about side effects to manage them etc.), but they felt these discussions were neither systematic nor were the responses recorded anywhere (*“I was asked ‘how are you, how have you been, how are you feeling’ ... but I didn’t fill in any forms”*), but they thought they could and should be (*“I wonder what they did record?”*). They shared no concerns about additional data collection placing a burden on them as patients, although there were some concerns expressed that this would increase the burden on healthcare staff (as noted above).

*“There is a lot of stuff to consider including the amount of paper that is generated, if it is all digital then it’s easy but then you are in the hands of coding errors. It takes a lot of empirical work to work up the right balance.”* (patient focus group)

While patients raised concerns about data sharing with pharmaceutical companies and

government departments, they were keen to access their own data, and shared varying experiences of obtaining their medical records, although they all agreed that data sharing between primary and secondary care providers was poor.

Patients need to be provided with a complete explanation of why their data is being used and how. The patients participating in the focus group raised concerns around their health data being shared outside of their healthcare provider, noting pharmaceutical companies and government departments in particular, especially if that data was sensitive or deemed to have been stigmatised.

*“It’s the drug companies that I think is the issue. One worries about commercial use of one’s data. Also, there is stigma. If you get lung cancer, you are a nasty old man who smokes and drinks and it’s all your fault. There is a stigma to different sorts of cancer, which is wrong. So you might not want that [being shared]. There are also concerns with data being shared between one government department and another, the home office and the police... which I think is worrying... The fact that it [health data] might be put to commercial use would worry me as well.” (patient focus group)*

The only point of contention raised by patients was the issue of non-compliance: what if a patient does not self-report their quality of life? Would these patients still be allowed the medicine? Participants thought it would be unfair to exclude them on this basis. They suggested that perhaps such questions could all be collected during a consultation, as *“I can’t imagine a review meeting with a clinician where a patient refuses to answer questions on how they are feeling or whether they’ve been able to wash themselves”*.

In summary, buy-in from patients is also fundamental. In our focus group, patients were supportive of an OBP approach on the condition they would know, and have control over, exactly how and why their data are being used.

## A clear and robust framework for information governance is required

Given the need for an OBP scheme to access, track, and link individual patient data across multiple datasets, a clear and robust framework for information governance is required, and must be agreed and put in place upfront with a trusted third party and with relevant data controllers.

This is because – for the purpose of assessing patient outcomes – data cannot be completely anonymised; tracking and linking of data will be required at the patient-level, using a unique patient-level identifier to link different data sources (using a pseudonym). This makes the data potentially identifiable (because the pseudonym could, at least in theory, be traced back to an individual person). Together with the potential “identifiability” of data, their health-related nature means that these data are also considered “sensitive”.

The UK Data Protection Act (UK DPA)<sup>29</sup> recognises a number of lawful reasons for processing sensitive personal data, one of which is the provision of health and social care. This provides the legal basis for keeping patient records in the NHS for direct clinical care. “Secondary” uses of NHS patient records, such as service evaluation or audit, or indeed an OBP scheme, are not explicitly recognised in the UK DPA and require a separate legal basis.

One such legal basis is explicit patient consent. However, obtaining explicit consent from all



individuals may not be feasible or proportionate, particularly in health care where the rationale for and benefits of data use (e.g. to monitor and improve health care and service provision) can be so great. Therefore, there exist various provisions in the law which grant a legal basis for the processing of sensitive personal data without explicit consent.

In the UK, this is generally through Section 251 of the NHS Act 2006. To attain Section 251 support, the purposes of the information recipient must be related to improving patient care, and must be in the public's interest, and will only be granted where it is either not possible or is too expensive or technically difficult to get consent from every patient. Whilst technical solutions may be available to collecting consent for OBP (e.g. if technological platforms are used for patients to self-complete quality of life data), the use of OBP at scale is unlikely to be compatible with obtaining explicit consent from every patient, especially as data from multiple sources will need to be linked, and the arrangements or sources may differ by scheme.

The question, then, is whether an OBP scheme could gain such permission to process sensitive personal data for the purposes of informing payment for medicines. There is precedence for this, through the ongoing implementation of the CDF, which is essentially a coverage with evidence development scheme administered by PHE through the CDF. Whilst the scheme operates at a population-level, individual patient-level data are required to assess the relevant outcomes and to link data sources. In order to maintain this special permission to process patient data, the NHS Health Research Authority's Confidentiality Advisory Group (CAG) must review this support on an annual basis, and very strict policies and procedures are in place which govern how data are collected, stored and released.

To implement an OBP scheme, the processing of sensitive personal data that are potentially identifiable will be necessary. This would require Section 251 approval to establish a legal basis. Provided that the appropriate policies and procedures were in place, it seems reasonable to assume that this approval would be granted, given that the use of patient data to support OBP may be considered compatible and aligned with other data initiatives for monitoring and improving the way cancer medicines are used and procured (e.g. through the CDF). However, this must be verified and agreed among the relevant stakeholders and authorities, and the processes in place would need to be reviewed on a regular basis.

Thus, in summary, given the need for an OBP scheme to access, track, and link individual patient data across multiple datasets, a clear and robust framework for information governance is required. Permission for patient data to be used in an OBP scheme should be obtainable provided the appropriate policies, procedures and safeguards are put in place.

## 3.4 Potential impact of OBP

### Case study: quantitative analysis

To understand the impact of a specific OBP scheme on costs and outcomes associated with a cancer drug for a defined patient cohort, a retrospective quantitative analysis was performed. Following several conversations with various parties around data availability and access, as well as discussions on the optimal use of available data, the project team decided to use *a particular drug under consideration for the treatment of previously treated advanced or metastatic cancer* as the subject of our case study. In order for this report to respect commercial sensitivities, we

refer to it as ‘Drug X’ and have removed specific details of the target patient population and literature references used for the basis of our model assumptions. Reasons for this choice of case study are described in Appendix 5.

Using the key clinical metrics published in the CDF report for Drug X supplemented by published clinical trial data for quality of life estimates, we generate a hypothetical patient cohort and used the OHE ‘OBP-Simulator’<sup>ix</sup> to quantify the potential impact of an OBP scheme on key parameters of interest, e.g. level of payment rebates for unsuccessful treatment and budget impact to the NHS.

One divergence between this case study and our proposed format of an OBP scheme is that the research indicated that an OBP would preferably be based on individual patient-level outcome data. As we did not have access to patient-level of data for this case study, we have mitigated this by simulating a cohort of 180 individual patients<sup>x</sup>, ascribing outcomes to those hypothetical individuals based on the population-level summary statistics obtained from the CDF report or trial data, constrained by the 9-month time window of data collection in the CDF. Details of the analysis are described in Appendix 5.

## Results of base case and scenario analyses

### To what counterfactual was the OBP scheme compared?

We compared the likely impact of the OBP scheme to a situation where no OBP would be in place and the drug would be routinely commissioned<sup>xi</sup> at a given price to the indicated patient population. Thus, if no OBP-scheme were implemented then for every patient the full nominal price of 100 would be due, regardless of treatment outcome. Hence, the impact of an OBP scheme against this counterfactual is limited to the number of rebates due and the resulting average per patient drug cost to the NHS.

### Base case analysis

In the base case we determined that all four core outcomes should be met. Hence, for every patient that 1) survives, 2) sees no disease progression, 3) has no toxicity leading to stopping treatment, and 4) returns to normal activities over the timespan of nine months, no rebate is due to the NHS and the manufacturer will retain the full price as paid upfront for the drug. If any one individual outcome is triggered, however, then the rebate would be due by the manufacturer to the NHS, which is set at 50% of the price.

Results in Figure 4 show that under such a base case, for 73% of patients one or more outcomes would be below the threshold, triggering the 50% rebate. On average per patient drug costs would then be 64% of full cost (or a 36% rebate to the NHS).

### Scenario analyses

Various scenarios, developed in discussion with the Steering Group, were analysed to consider different outcomes to be met as well as alternative inputs for the OBP-price and rebate

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<sup>ix</sup> Excel-based simulation model created for this project.

<sup>x</sup> This sample size is in accordance with the number of patients included in the study undertaken in the CDF.

<sup>xi</sup> Note that we refer to this counterfactual as ‘routine commissioning’, but this could be through baseline commissioning or for a limited time through the CDF. The key point of differentiation is that the medicine is made available at a single, uniform price.

percentage. Scenarios 1 and 2 show the impact of an OBP in which, instead of all four outcomes, only a selection of core outcomes would need to be met.

- Scenario 1: OBP-scheme requiring survival (S), disease progression (DP), and toxicity (T) outcome to be met.
- Scenario 2: OBP-scheme requiring survival (s) and disease progression (DP) outcome to be met.

We also considered a scenario 3, which is the same as the base case except that the starting price under the OBP would be 120 while the price without the OBP-scheme remains 100.

Results for scenarios 1-3 are presented in Figure 4 and show that the average per patient drug costs to the NHS is expected to be lower with this OBP scheme compared to routine commissioning under all scenarios.

The effect of reducing the number of thresholds for ‘successful’ treatment (scenario 1 and scenario 2) is to gradually reduce the proportion of patients for whom a rebate is due, as expected, and hence gradually increase the average per patient drug cost to the NHS under such schemes. Scenario 3 shows that a higher price under the OBP leads to higher average drug costs per patient compared to the routine commissioning scenario (where OBP price = no OBP-price) but still lower than without OBP. Further details are shown in Appendix 5.



Figure 4: Results - base case and scenario analyses

Finally, we show in Figure 5 how the average (mean) drug cost per patient changes with the upfront agreed rebate percentage, assuming all four core outcomes need to be met. This shows that at a low rebate percentage the mean drug costs per patient approach those of non-OBP costs, while at a high rebate percentage these costs would be reduced to just a fraction of non-OBP costs.

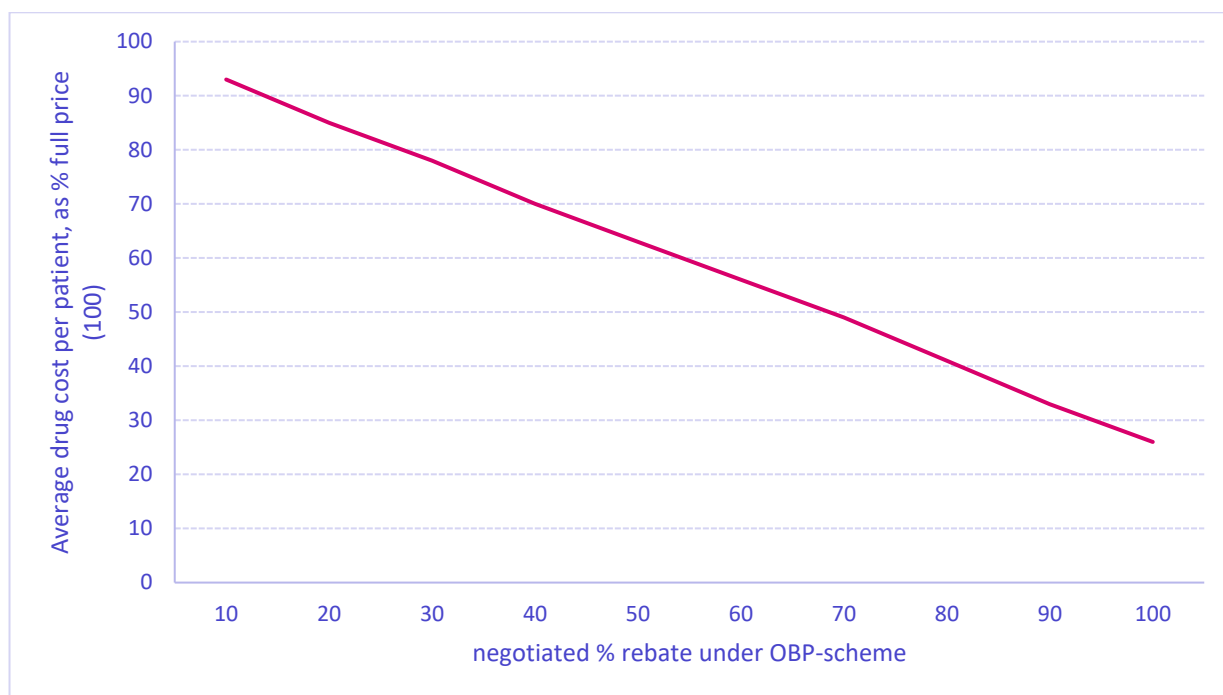


Figure 5: Average (mean) drug cost per patient as function of rebate percentage

Finally, given a combination of a low percentage rebate and a price for successful treatment under the OBP greater than the price in routine commissioning, average per patient drug costs could even be higher compared to routine commissioning.

## Limitations and learnings

There were some limitations to our data inputs, most notably having access to population-level estimates only. Full access to the SACT data would permit:

- 1) individual-level analysis and linked patient-level data for each outcome, and
- 2) analysis according to an OBP scheme time-horizon observed at the patient-level (for the current analysis, we were limited to a 9-month window of data, with the follow-up time for some patients being shorter (minimum three months)).

Noting these issues serves to **highlight the data requirements for a real-life prospective OBP scheme**, where, as our research suggests, preferably individual-level patient data would need to be captured.

In a real-world pilot, we envisage that NHS Digital (previously PHE) could be instrumental in providing individual-level data which are uniformly captured at the NHS level (rather than in different ways at the various local levels), as noted in section 3.1. The development and roll out of the PHE health-related quality of life measure would be critically important to also capture those measures in addition to survival and disease progression (proxies). Without this or a similar (local) initiative, patient-reported outcomes would still need to come from another source and then be linked at the individual patient level.

A limitation of the scenario described is that, in fact, **OBP can most plausibly generate benefits for all of patients, payers and manufacturers** in cases where it is used to facilitate **earlier patient access** to a likely cost-effective but as yet uncertain new treatment. In such cases, the

counterfactual would actually be no (or delayed) patient access to the drug. While we were not able to show this using a retrospective example (which requires data of use in practice), we have demonstrated that, compared with routine commissioning at a single price, OBP could offer more control of medicine spend at the individual patient level based on realised outcomes. Where this offers the opportunity for accelerated or improved medicines access, the OBP would then be expected to have a (positive) impact on patient outcomes as well as (short term) manufacturer revenue, while allowing the NHS to provide timely access and mitigating their financial risk. While this is not demonstrated numerically through this case study, it is also important.

Another limitation is that we have not assessed the full financial impact of implementing an OBP arrangement, which would need to include the costs associated with setting up and running the OBP scheme. We recommend that further information on these costs be collected as part of the next phase 3 research.

Finally, we note that the analyses presented consider the context of potentially cost-effective yet uncertain treatments for patients with cancer, where the expectation is that payments linked to further real-world evidence on the drug is an appropriate and feasible way to mitigate the uncertainty. While the principles and direction of findings would likely be generalisable to other cancer and non-cancer conditions (at least for similar schemes and comparators), the exact results are not and different considerations about the most appropriate design of an OBP in other contexts will influence this.

# 4 Conclusions and recommendations

This Phase 2 research study builds on outputs of our Phase 1 research study, the findings of which indicated that the wider use of OBP in the NHS is possible and desirable for some cancer medicines, including new treatment options or existing medicines used for new indications. The Phase 1 study recommended four core patient-centric outcomes which should be captured through the OBP scheme: (i) survival; (ii) disease progression; (iii) long-term treatment side-effects; and (iv) return to normal activities. It also highlighted some challenges, including: (i) the timeliness and quality of real-world data and (ii) the need to be clear on the benefits of OBP to patients, the NHS and industry.

The Phase 2 research study is designed to build on this through identifying key practical barriers to implementing OBP. Using a range of investigative techniques and analyses we:

- Narrowed down to a recommended form of OBP
- Provided greater clarity on available data to support an OBP scheme, including both data that is currently available and that which is potentially likely to be in the short-term
- Provided a clearer understanding of operational issues and burdens associated with the implementation of OBP schemes
- Modelled the potential financial impact of an OBP scheme to the NHS, demonstrating that OBP can reduce the risk to payers from uncertainty about the cost-effectiveness of cancer medicines.

We find that OBP offers a ‘win’ for patients, in cases where simple pricing approaches would mean delayed or restricted patient access. Expediting the pricing agreement process consequently benefits the NHS and industry. We have noted that the CDF already does something similar by offering patient access over a period of further data collection. However, where further timely clinical evidence development is unfeasible, OBP could offer a route to faster or more comprehensive patient access in particular where uncertainty remains about a medicine’s clinical effectiveness even once clinical trial data are mature and no further data are expected.

Findings suggest that it is not currently possible to collect the data required for an OBP scheme incorporating all four outcomes as set out in our Phase 1 research study.

In the short-term, an OBP scheme based solely on outcomes of a more clinical nature may be feasible, though this would not capture the quality-of-life outcomes and hence may fail to fully reflect the drug’s value to patients.

However, ongoing developments, further data collection initiatives and future activities based on our recommendations could arguably create conditions necessary for such an OBP scheme to be agreed and conducted with success in the future.

We have taken a pragmatic approach to OBP, focusing on what could be done to implement such a scheme in the near future, whilst creating a platform for future research and activities

which is capable of robustly identifying whether OBP schemes are a viable option for the NHS in England. With further investment by NHSE&I in data capabilities, more sophisticated forms of OBP are conceivable.

## 4.1 The outlook on data availability for OBP in the NHS is positive but with some way still to go

The advancement of electronic health records and national datasets, as well as improved data linkage opportunities mean that the capture of clinical metrics is relatively well advanced in the NHS. As a result, data on survival and disease progression are mostly available and can be leveraged for an OBP scheme.

In the short-term, a realistic and pragmatic local-level OBP scheme in the short-term could be based on survival, treatment duration and toxicity data captured through existing electronic health records and e-prescribing systems whilst being mindful of new emerging data for the remaining outcomes.

Although the landscape for collecting patient-reported outcomes is evolving, capturing data on long-term side-effects and return to normal activities of daily life is a critical barrier that needs to be addressed for OBP schemes to become viable in the long-term.

To further support collection of outcomes data and ensure it is efficient, standardised approaches should be established. For clinical measures, standardised templates and reminders could be set up for clinicians and nurses to report data. For patient-reported outcomes, this could be through the creation of a patient portal. In addition to collecting the necessary data in a standardised format that can be used for OBP, mechanisms to clean the data and link these datasets on the individual patient-level need to be considered.

Importantly, while patients are strongly in favour of patient-reported outcomes being collected by the NHS, they raised concerns around their health data being shared beyond their healthcare provider. For patients to support such data sharing, they need to be provided with a complete explanation of why their data are being used and how these are processed for the purpose of an OBP.

Engaging in an OBP scheme is expected to provide an (additional) incentive for stakeholders to improve data collection. Yet, ensuring that all parties involved are aware of why the underpinning data should be collected and the benefit this could bring to patients was considered essential. While some NHS staff interviewed had concerns about the time investment required to collect and collate additional data on patient outcomes, there was nevertheless cautious support for this in order to support OBP.

## 4.2 Non-data challenges relate mainly to stakeholder buy-in and the rebate process

Beyond data collection requirements, OBP requires dedicated staff and processes to implement and oversee the scheme(s). Although the positive patient outcomes expected from OBP are intrinsically important, there are financial costs associated with setting up and running OBP,

and the burden that thereby falls on hospitals needs to be recognised.

Critically, the details of the OBP rebate process need to be clarified up-front to avoid a potential misalignment between who pays for the treatment, who receives the rebate and who pays for the cost of data collection to facilitate the OBP scheme. Currently there is uncertainty around identifying the final beneficiary of the rebate, as the NHS trusts administer the treatment and procure the medicines, yet ultimately the commissioning body pays for the medicine. Additionally, completing and processing requests for rebates (in case of unsuccessful treatment) in a timely manner is expected to place an additional burden and might better be coordinated centrally, e.g. by NHSE&I.

### 4.3 Linking outcomes to payments in an OBP scheme needs to balance simplicity with individual level impact

The Phase 2 research study results indicate that an OBP scheme should ideally be based on treatment outcomes for individual patients rather than average outcomes across populations. Stakeholders also agreed that a simple binary payment would be preferred for now, i.e. one level of payment if the treatment is successful for an individual patient, and a lower payment if the treatment is unsuccessful for that patient.

For practical reasons, the initial price paid for the medicine would likely be that which corresponds to a value-based price under the assumption that the treatment is effective, with a subsequent rebate if outcomes turn out to be poor.

Rather than a composite outcome measure (which is complex to establish and negotiate), we suggest that success threshold levels should be set for each of the individual outcome measures separately. Failure to meet the threshold level for any one of the outcome metrics would be taken to indicate unsuccessful treatment for the purposes of reimbursement. Manufacturers would need to prepare for that as the current development model may not be based upon maximising response on all of these outcome measures.

For each outcome of the OBP scheme, the threshold for “success” for each outcome should be determined and negotiated for each scheme separately. It is not possible to set rules that will apply to all medicines. However, the principles of how to establish and agree the appropriate thresholds for treatment success – as well as the level of rebate that is applied when they are not met – could be investigated in the next phase.






The OBP scheme tested in this research study is expected to reduce the average per patient medicine costs to the NHS, compared to routine commissioning (with a single price) under all scenarios analysed. This will depend on various OBP design characteristics such as the: (i) rebate percentage; (ii) the price of the medicine using an OBP scheme versus without using an OBP scheme; and (iii) threshold level of each outcome considered for the rebate. A comparison of the potential impact of an OBP with the potential burden of collecting and using data on these outcomes will help to balance the simplicity, and hence practical feasibility, of the scheme.



## 4.4 Key characteristics of an OBP scheme

The characteristics of any OBP scheme should be agreed between the NHS and the drug's manufacturer as part of the commercial negotiation process. However, a plausible set of 'default' characteristics for a scheme (outlined below) have been identified during this research, to be adapted as appropriate in individual cases, recognising the importance of simplicity and practicality. More complex forms of OBP are conceivable in the longer-term, but these suggested characteristics represent a pragmatic first step.

A default set of characteristics for future OBP schemes could specify the following:

-  Prices and rebates are linked to treatment outcomes for individual patients, rather than average outcomes across populations
-  A two-level price, with an initial payment by the NHS and a subsequent rebate to a pre-agreed lower price level if the pre-agreed, expected 'success' levels of patient outcomes are not met
-  'Successful' treatment is defined as where patient outcomes meet all of the separate threshold levels set for each of the individual outcome measures
-  Outcome 'thresholds' indicative of treatment success can be based on available clinical trial data and available real-world-data for an outcome metric, but should also align with points identified in NICE's HTA process that determine cost and clinical effectiveness
-  The scheme should use all four outcomes types identified in Phase 1, with omissions agreed between all parties on a case by case basis. Not already having NHS data readily available should not on its own justify omitting an outcome type

NHSE&I should recognise the specific value of OBP in cases where data collection from completed clinical trials or managed access arrangements has failed (or is unlikely) to resolve clinical and cost uncertainties. In line with findings from Phase 1 of this research OBP schemes would be best suited to medicines with the criteria listed below:



Substantial uncertainty remains about a medicine's clinical- or cost-effectiveness even once clinical trial data are mature (this may particularly be the case where there is high heterogeneity of treatment effect among patients)



The medicine's use is clinically desirable, and plausibly cost-effective but characterised with decision uncertainty



There is a small to mid-sized NHS patient population, with patient groups small enough to allow for manageable follow-up, but large enough to justify the resource burden of operating an OBP scheme



The cancer being treated is of a type where improvements in the clinical or quality of life outcomes of NHS patients receiving the medicine can be observed within one or two years, and data on those outcomes can be captured at scale within the NHS in the same timeframe

## 4.5 Piloting a local OBP scheme

Collection of clinical outcomes is relatively advanced and could serve as a starting point for OBP schemes, while data linkages between these and the other core outcomes can be established through pilots and at scale, as data collection initiatives are evolving for ‘long-term treatment side-effects’ and ‘return to normal activities’. Although it is not currently possible to ‘fully’ pilot an OBP scheme with a complete dataset for each of the four core outcomes, data availability and linkage should not be considered static. As the data landscape is changing, pilots capturing just clinical outcomes or all core outcomes, including the less developed ones, provide a valuable learning opportunity to inform and implement a more complex OBP scheme in the future.

A pilot study with available data could offer a ‘proof of concept’ of the model developed during phase 2 and provides the opportunity to ‘test’ key research questions such as those outlined in box 4. These questions offer a preliminary structure for what evidence could be collected in a potential pilot trial and are to be developed in future research. With clear success criteria, the pilot would provide further evidence to understand whether a national OBP scheme would be viable. Implementing the recommendations below would create an opportunity for an OBP scheme to be more comprehensively tested at a national level (if deemed necessary and viable), and to further the evidence base. Addressing the challenges listed in the recommendations and implementing the learnings from the pilot trial could potentially accelerate patient access to certain new medicines. If a pilot were to be undertaken, it could comprise of the following initial stages and preliminary research questions:

### STAGE 1: Negotiating an OBP scheme

- The involved parties should discuss and agree with NHSE&I a (selected set of) medicine(s) to prospectively pilot OBP considering the type of medicines (e.g. one-off or multiple cycles of treatment,) the dynamics of patient pathways and the availability of other new treatments.
- NHSE&I should open discussions with the corresponding manufacturer(s) about an OBP scheme for their medicine, including how to share the costs of additional data collection.
- The design should aim to find a good balance between simplicity and potential impact by incorporating outcomes that matter most to patients. The extra burden must translate into better outcomes for patients through timely treatment availability and/or de-risking the investment to the payer.

- The threshold setting should be medicine- and context- specific and should consider the counterfactual, whether that is routine commissioning or no (or delayed) access.

#### STAGE 2: Implementing the OBP scheme

- Where possible, routinely collected data should be used.
- Investment to collect data on outcomes measures that are not currently routinely available and to optimise completeness and quality of all data used.
- A clear process needs to be established for linking observed outcomes to the appropriate financial implications and obtaining rebates, if due.
- Appropriate information governance needs to be assured.

#### Box 4 – Key questions raised by our research to be ‘tested’ in a pilot

- What factors make successful agreement of outcomes and thresholds to be included in the scheme more likely? Can any guiding principles be agreed?
- What is the optimal organisation and governance of OBP schemes, and administration of the rebate process, from an NHS operational perspective?
- What are the extent and cause of low-quality or missing data across each outcome, and how can this be minimised?
- What are the challenges and practical solutions for linking individual patient level pseudonymized data from distinct datasets (e.g. SACT data with quality of life data), both operationally and from an information governance perspective?
- How much additional time is required for patients/clinicians to record valid and reliable quality of life data?
- What are the costs at a Trust level associated with setting up and administering OBP, and who would be responsible?
- How much time is required to chase up rebates where these are due?

## 4.6 Policy Recommendations for working towards a national OBP scheme

The below recommendations would support the implementation of more complex OBP schemes in the long- term if this were to be taken forward nationally.

1. A local-level pilot of an OBP scheme based on data available for clinical outcomes (survival, disease progression, and short-term treatment side effects) would be feasible and desirable. Relevant stakeholders should convene to discuss and agree how a pilot could be taken forward.
2. NHSE&I should recognise the potential benefits of OBP. This includes (i) the potential for patients to have access to some new medicines sooner than with a simpler pricing scheme (ii) and ensuring that the NHS pays only for the outcomes actually achieved for patients. Data collection should be encouraged for this purpose.
3. To minimise data barriers to an OBP scheme of the kind set out in our phase 1 research study, NHSE&I and NHS Digital should invest to:

- Optimise data collection to more precisely capture disease progression/ relapse/recurrence in the SACT dataset.
  - Allow patients to report long-term side effects and their ability to return to normal activities through PROMs data collection infrastructure (for example via online patient portals).
  - Optimise the quality and linkage of data from the Cancer Quality-of-Life Metric Project and introducing flexibility around the time points that data are captured post-treatment.
4. Where OBP schemes are implemented, dedicated resources should be made available to support the underpinning data collection. This could include:
- Funding for local NHS Trusts to employ data teams, and for NHS Digital to support the quality and completeness of Trust-level data collection (and analysis of that data).
  - Resource to ensure a trusted third party (potentially NHS Digital) can collect patient-reported outcomes data at scale and link these to individual-level patient data on clinical outcomes (including data already collected through SACT).
5. The Department of Health and Social Care, in conversation with Trusts and industry, should further investigate the optimal organisation and governance of OBP schemes, including data governance and the process by which any rebates might be paid by manufacturers to the NHS.

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