

MAKING OUTCOME-BASED PAYMENTS A REALITY IN THE NHS

RESEARCH STUDY: PHASE 1

1.0 INTRODUCTION

Cancer Research UK (CRUK) believes cancer patients should have access to the best, evidence-based innovative cancer drugs. Today advances in drug development are offering progression-free survival, better quality of life and hope to cancer patients. Yet, translating these medical advances into routine access for NHS patients remains challenging. Compared to the other G5 countries patient access to new cancer drugs in the UK remains slower.

With the establishment of the new Cancer Drugs Fund in England there is greater focus than ever before on data collection and its use in decision-making. As the NHS continues to face unprecedented financial challenges it is essential future spend on cancer drugs better reflects patient outcomes and experiences in clinical practice. Clinical trials remain the gold standard way for researchers to collect evidence for new treatments, so we can continue to improve cancer treatments and make them available to patients. We want to build on trial outcomes, with recognition of the role played by evidence in clinical practice to support access. This is especially true as patient populations are segregated into ever smaller patient groups for new precision treatments.

A recent independent report, the Accelerated Access Review (AAR) emphasised the need for the NHS in England to enhance the pull through of innovation into the NHS improving patient access to new drugs. As part of a new approach to accelerate innovation, the AAR calls on NHS England to adopt flexible pricing mechanisms to pay for transformative technologies and medicines.

CRUK believes one way of supporting NHS England (NHSE) to fulfil this broad vision is to develop a new model of paying for cancer drugs. In partnership with Greater Manchester Health and Social Care Partnership we are looking to pilot the feasibility of an outcome based payments scheme in the NHS. To achieve this a three phased approach will be carried out.

The first phase of the research will be to define the outcome criteria. The same criteria will then be used in the next two phases of this study – retrospectively testing out the criteria using existing datasets, with the final stage using the criteria to carry out a live pilot within Greater Manchester (subject to an assessment of viability following stage one and two).

Current decision making

At present the NHS pays a price for a new drug based upon NICE recommendations made at a single point in time, increasingly based on immature data. For many innovative cancer drugs this means the NHS is currently agreeing a price on data that hasn't yet been tested in a real world setting, especially with regards to longer-term outcomes including progression-free survival and overall survival. In an outcomes-based system, where RWE is collected from unselected patient populations in the context of real clinical practice, a price can be aligned directly to the most recent data on patient benefits. The price can be increased or decreased based on maturing data, including progression-free survival and overall survival as well as patient quality of life.

Real-world evidence

Cancer Research UK and Public Health England recently demonstrated how RWE from clinical practice add important insights that complement clinical trial data in a world-first analysis based on the national Systemic Anti-Cancer Therapy data set (SACT). The dataset, introduced in 2012, routinely collects information on the treatment of all cancer patients in England on an ongoing basis. This first analysis included 23,228 breast and lung cancer patients across England, and explored the proportion of those patients that died within 30 days of their last cycle of chemotherapy. Importantly, upcoming analyses will explore outcomes from treatment with specific cancer drugs, allowing assessment of ‘real-world’ benefits of those drugs in a national, unselected patient population – a key strength of the data set.

We believe with our expertise and experience with the SACT dataset, and our existing relationships with drug companies (from partnering on clinical trials) CRUK can play a crucial role in testing out a new approach to the pricing of new emerging cancer drugs in the NHS.

Phase 1

CRUK are looking to fund the first phase of the study which will involve undertaking a comprehensive review of the literature to find out what outcome criteria are currently being measured in real world settings globally, including where outcomes based payments systems are already in place. This phase will be complemented by conducting qualitative interviews with patients, clinicians, and commissioners, as well as other key stakeholders with the aim of developing ‘outcome’ criteria that can reflect the value of new drugs through the use of real-world evidence (RWE). These criteria will consider patient outcomes, including considerations for quality of life, years of life lost and loss of productivity. The criteria should also move beyond clinical outcomes and capture, where possible, additional quality of life measures, such as return of function to usual activities (e.g. work / exercise), independence (from carer support) and economic activity.

A steering group including (but not limited to) representatives from NHS England, National Institute for Health and Care Excellence (NICE), the Department of Health (DH), pharmaceutical industry and patients will oversee all phases of the study. A pharmaceutical partner (tbc) will be involved in the management and funding of the final stage of the study, the live pilot.

The body of evidence generated during this project will inform and influence the Department of Health, NHS England and NICE. Depending on the results of the study we can provide recommendations on whether to consider outcome based payments as a flexible pricing mechanism that could be adopted across the NHS in England.

2.0 AIMS

Cancer Research UK would like to receive proposals/tenders to carry out the first phase of the study, defining the outcome criteria to be used in an NHS setting.

This first phase will:

1. Produce a body of evidence that can underpin criteria to evaluate the real-world clinical benefit of a new cancer drug – criteria that can then be used to adapt payments.
2. Define the criteria, which will provide a benchmark for future outcome based payment schemes for cancer drugs with cancer specific measures. Taking into consideration future opportunities to use data to feed into NICE HTA appraisals and re-appraisals.
3. Be pragmatic and produce evidence that is transferable to a clinical setting in the NHS, and can be used to improve patient outcomes and drive value for the health service – taking into consideration the existing capabilities of the SACT dataset, as well as the potential for future indicators to be developed (e.g. Quality of Life metric).

3.0 METHODOLOGY DETAILS

CRUK will fund the first phase of the study, to define the outcome criteria for which we will use in the following two phases of the study.

This stage of the study will include both a comprehensive literature review and qualitative interviews to gather relevant perspectives on the proposed outcome criteria. The literature review will offer current UK context and international case study comparators on which the outcomes will be based. Qualitative interviews may be carried out with patients, clinicians and other key stakeholders, including members of the pharmaceutical industry, NHS England's new commercial unit and the SACT team to gather perspectives of what the outcome criteria should include. Patient access for interviews can potentially be gained through CRUK's extensive patient network. Ethical approval will need to be obtained from the hosting higher education facility. The Principle Investigator (PI) will have responsibility in obtaining ethical approval for this study.

The evidence gathered through the literature review in conjunction with interview data will produce a final criteria from which the second and third phases of this study can be tested within an NHS setting.

Your proposal should outline the following:

- Detailed methodology proposed for both the literature review and interviews
- How you would recruit research participants for this study
- Guidelines and procedures in place, including ethical approval
- Confidentiality & Data Governance
- Quality control mechanisms
- CVs of all staff working on this study
- Roles and responsibilities of each staff member, including project lead and main point of contact

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- Details on any work that will be sub-contracted
- Any potential challenges/risks in undertaking this study
- Multiple options of what could be included as part of the study with a breakdown of costs

4.0 Outputs

Outputs from this phase 1 of the study should be a comprehensive report:

- Showcasing the key findings, literature review including most comparable case studies and key themes from the qualitative interviews and analysis undertaken.
- As assessment of similar case studies using international examples
- A value framework built on patient reported outcomes and experiences which can be used to measure the real clinical value of new cancer drugs. This criteria will be applicable to an NHS setting.
- Conclusions/recommendations detailing current datasets that can be used to measure the outcomes as defined in the criteria and what/if new datasets need to be created.

This list is not exhaustive and additional outputs will be considered. The research can be made available in the public domain through the publication of academic papers.

5.0 BUDGET

Applicants should be sure to demonstrate value for money. Please provide a detailed breakdown of the cost of each discrete element within your proposal to provide options for the Cancer Research UK/ Greater Manchester Partnership steering group to fund. The combined budget available for phase I is anticipated to be up to £100,000 (inclusive of VAT/sales tax) in total.

6.0 TIMESCALE

Please respond with expressions of interest as soon as possible. A full proposal giving a detailed methodology and budget breakdown should be submitted by close of business **8th September 2017**.

All proposals will be independently peer reviewed w/c **11th September 2017** and the decision will be made by the end of September. The chosen applicants will be invited in **October 2017** to finalise the project plans and key deliverables and to discuss contract terms with a view to starting the study by **November 2017**. The qualitative interviews will commence once ethical approval has been granted.

7.0 SUBMISSION DETAILS

The upper most budget for both the literature review and qualitative interviews is £100,000 (inclusive of VAT/sales tax). Collaborations are encouraged in order to ensure the highest quality research is

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carried out for both components for this phase of the study. CRUK is not bound to accepting the lowest bid.

Your outline should include:

- Introduction/Background
- Ethical approval and governance procedures
- A detailed methodology
- All intended outputs
- Timelines
- Breakdown of the budget per topic (costs should include VAT where applicable)
- Expertise relevant to the study
- Governance structures at the organisation
- Perceived risk and the mitigation steps that will be taken

8.0 TENDERING CRITERIA

Proposals will be considered on the following criteria:

1. Method of investigation
2. Track record of lead applicant/research group
3. Support/ resources needed
4. Approach for involving patients to inform the outcome criteria
5. Application to real world setting (English NHS)
6. Proposed costs (value for money)

Please send your project outline by **5pm on September 8th 2017** to Lucie Hooper, Policy Research Manager, Policy Department– lucie.hooper@cancer.org.uk; Tel: 020 3469 6349