
Drug evaluation

Position Statement from The Institute of Cancer Research, London

Summary

The Institute of Cancer Research (ICR) strongly believes that drug evaluation systems should be designed to provide cancer patients with access to innovative and effective treatments as quickly as possible. The ICR supports the role of NICE in making difficult decisions over which new medicines the NHS can afford to offer, but believes its current approach is failing to provide patients with fast access to the best new treatments. We need urgent reform of NICE's system for drug evaluation, to unblock the bottleneck in the flow of innovative and effective cancer drugs to NHS patients.

The ICR believes that the UK needs systems for the appraisal of drugs and technologies that are led by clinical evidence but also properly take into account and prioritise innovation. We need to incentivise the discovery and development of exciting, innovative new cancer medicines that work in brand new ways, on their own or in combination, to overcome drug resistance. We should be moving our focus away from 'me too' drugs and on to novel treatments and those for diseases where there is unmet need. The ICR believes the process for evaluating treatments must take much more account of the importance of innovation in cancer drug discovery to build confidence among researchers and companies that their efforts to innovate will be rewarded. We also believe that NICE's calculation of cost-effectiveness does not sufficiently capture some of the broader aspects of a drug's value, such as its longer-term impact on quality of life and whether it can be targeted at specific groups of patients.

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Background information

Healthcare systems such as the NHS work to limited budgets and are constrained in the treatments they can afford. They need to employ evaluation processes for drugs and technologies to make decisions about the range of treatments that should be made available, based on what will deliver the greatest benefit for patients within a constrained budget.

In England, the evaluation of the drugs and technologies to be offered by the NHS is the responsibility of NICE, which makes judgements about whether it would be a good use of public resources to make a new treatment available for patients. NICE is respected internationally for its rigorous examination of evidence, but has been unable to prevent numerous controversies over the availability of innovative new treatments at a price that the NHS can afford.

The appraisal process NICE uses for new drugs and technologies considers evidence from clinical trials and peer-reviewed research, economic evidence on the cost of treatment, and the views of patients, clinicians and stakeholders. NICE uses the price per quality-adjusted life year (QALY) to determine how cost-effective each treatment is. It applies an upper limit for recommending treatments of £20,000 per QALY for drugs, with the option to raise this to £30,000 for drugs that are considered innovative, and £50,000 for drugs used for patients at the end of their lives.

In 2013/14, the Government proposed a new system of 'value based' appraisals, to broaden the assessment criteria for new drugs and technologies to include their impact on wider society and the lifetime burden of disease. The ICR expressed concern over the proposed new processes, arguing that, while well intentioned, they risked having unintended consequences. They could, for instance, have created disparities in the number of drugs made available for children and adults, and reduced the number of new therapies approved overall – hindering the development of innovative cancer treatments. NICE decided not to implement the proposals after concerns were raised during a public consultation, but it did commit to reform of its processes for drug evaluation.

In 2016, NICE took over responsibility for the Cancer Drugs Fund (CDF), as a means of making the fund more evidence based and financially sustainable. The Government originally set up the CDF in 2010 to give patients access to cancer drugs which had not been approved by NICE, but increasing demands on the fund had rendered it unsustainable. Under the new NICE management, there has been early evidence of improvement, including examples of constructive dialogue with the pharmaceutical industry.

In 2017, NICE and NHS England introduced a budget impact threshold, where NICE engages companies in discussion where drugs are likely to cost the NHS £20 million or more a year in any of their first three years. The introduction of new drugs could also be phased in over a longer period to prevent disrupting to the funding of other services.

Key ICR positions on drug evaluation

- The ICR strongly believes that the drug evaluation system for the NHS should be designed to provide cancer patients with access to innovative and effective cancer drugs as early as possible after they have been licensed.
- The ICR believes that the UK needs a system for the appraisal of drugs and technologies that is led by evidence but that also properly prioritises exciting, innovative new medicines, particularly those for diseases where there is unmet need. We need to incentivise the discovery and development of innovative new treatments that work in brand new ways, on their own or in novel combinations, to overcome drug resistance.
- The ICR supports the role of NICE in ensuring public resources are spent on treatments that are good value for money, but believes its systems for evaluating new medicines are in urgent need of reform. We support NICE's evidence-based approach and the comprehensive nature of its data analysis, but its processes can be slow and inflexible, and have not kept pace with advances in science or sufficiently stressed the importance of innovation.
- The ICR believes the process for evaluating treatments must take much more account of the importance of innovation in cancer drug discovery. Regulatory agencies such as NICE need to change their definition of innovation to prioritise truly innovative treatments, tackling cancer in new ways, and which have the potential to deliver the step changes in outcomes that we need to see. The evaluation process must assess whether a cancer drug is novel in its design or its drug target, unique in a rare disease, or innovative in the way it is used or delivered. While we think that many drugs are priced too high, we believe NICE should have the flexibility to accept a cost-effectiveness ratio of more than £30,000 per QALY for drugs that are regarded as particularly innovative and effective.
- It is critical that NICE does not disadvantage certain groups of patients. For treatments for rare and paediatric cancers it is difficult to collect the level of evidence that NICE requires to demonstrate cost-effectiveness. We believe the NICE technology appraisal methodology needs to adapt to meet the challenges of evaluating treatments in smaller patient populations.
- NICE needs to be flexible in assessing treatments on the basis of data from smaller, smarter phase II trials – rather than waiting for large-scale phase III trials – as a means of getting treatments to patients more quickly. Increasingly, regulators are approving drugs on the basis of phase II trials to speed up their development, but NHS patients will only benefit if NICE is able to assess the same evidence in its appraisals. NICE needs to more often assess drugs on the basis of incomplete survival data, or use other

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measures of clinical effectiveness such as progression-free survival. For rarer cancers, NICE should accept results from small trial populations and observational data.

- We believe NICE's calculation of cost-effectiveness is missing or under-weighting some of the broader aspects of a drug's value. NICE appraisals should take into account other aspects of a drug's value – such as whether the drug delivers benefits to patients where there are no existing effective treatments, whether it can be used in combination with other treatments for greater benefit and whether it could be targeted at particular groups of patients using biomarkers, as well as its degree of innovation and effectiveness and any improvements in the health-related quality of patients' lives.
- The ICR supports NICE's end-of-life criteria, which allow the cost-effectiveness threshold for drugs to be loosened for treatments benefiting patients at the end of their lives. We believe these criteria have played a vital role in getting innovative new drugs to patients. Approval of a treatment at the end of life has often been a stepping stone to more general access on the NHS, and has helped build up a bank of cancer drugs that can be used in combination with each other for greater patient benefit. However, there have been instances where drugs that have been approved under the end-of-life criteria are then not later made available to patients for earlier treatment despite showing significant benefit. In such cases, NICE should work with manufacturers and The Department of Health to explore options for making treatments that have been shown to be effective earlier in treatment more widely available.
- We need to ensure that cancer drugs are made available for use on the NHS in combination with other drugs and curative treatments such as radiotherapy as means of overcoming resistance to treatment. Once a drug has been approved, there may be few incentives for the manufacturer to trial it in combination, leaving the burden of running these trials with academic institutions. It may be possible to make early drug approval conditional on future inclusion in rational combination studies, as one way to ensure companies run the combination trials.
- We believe that the introduction of a budget impact test needs to be carefully monitored, to ensure it doesn't slow access to cancer drugs for some patients. We support NICE's role in ensuring NHS resources are used effectively, and accept that the budget impact test could be useful in managing availability of treatments at a time when resources are tight. But we believe that the test could disadvantage some groups of patients, such as those with common cancers where demand is likely to be high, and would urge NICE to reconsider should that prove to be the case.