ICR The Institute of Cancer Research

Early access to medicines

Position Statement from

The Institute of Cancer Research, London

Summary

It is critical that patients with cancer have access to innovative drugs and treatments as early as possible. A pragmatic and flexible regulatory system is needed in the UK and Europe that reflects the changing scientific realities of cancer and its treatment.

We need to build on recent efforts to speed up access to innovative treatments. We can do this in part by generating evidence of a medicine's safety and efficacy more quickly through greater use of innovative trial designs, making use of biomarkers to direct treatments at defined patient populations. We also need a more flexible approach to licensing, to speed up the regulatory process. Initiatives must also promote greater use and uptake of repurposed and off-patent medicines.

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Background information

Bringing innovative drugs and treatments to patients with cancer is a lengthy process. It takes on average 12-14 years to bring a new cancer drug to market from the point of patenting through to licensing.

In recent years, regulatory agencies have introduced several schemes aimed at accelerating drug approvals based on more flexible standards of evidence, such as the UK's Early Access to Medicines Scheme (EAMS), where patients get access to medicines before full market authorisation has been granted. At the same time, initiatives such as the EU's PRIME scheme offer pharmaceutical companies a streamlined regulatory route for treatments targeting diseases where there are currently no effective options.

In 2014, the UK Government commissioned the Accelerated Access Review with the aim of making it easier for NHS patients to gain access to innovative medicines, medical technologies, diagnostics and digital products — and to improve efficiency, care pathways and outcomes for patients. The Department of Health published its full report in 2016, proposing improving horizon scanning to identify innovative treatments and a transformative designation for products with the potential for greatest impact to accelerate their path to market – potentially by up to four years. This pathway launched in April 2018.

Despite this increased focus on early access, there is still much more to be done to accelerate development of the most innovative cancer drugs. Researchers are increasingly classifying cancers into many different smaller subgroups as our understanding of cancer genetics and biology advances. Regulatory systems therefore need to show more flexibility in the types of evidence required to approve drugs for use in small populations**,** and to address barriers to the use of novel trial designs.

This position statement addresses what can be done through the licensing system to ensure earlier access to innovative cancer treatments. In addition to this, we need to ensure that, once approved, drugs are quickly made available to patients through health systems such as the NHS. We address the availability of drugs on the NHS through the ICR's separate position statement on drug evaluation.

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Key ICR positions on early access to medicines

- The ICR believes it is crucial that innovative treatments are made available to patients as quickly as possible. It is critically important that we have a range of treatments available for use to combat the major challenge of cancer drug resistance, which will increasingly require drugs to be used sequentially or in combination.

- We believe the regulatory and licensing system should be both robust but also flexible and pragmatic enough to allow earlier access to innovative treatments. Current initiatives that promote a flexible and progressive approach to licensing such EAMS and PRIME and the proposed 'transformative designation' in the UK are a step in the right direction. We support licensing based on conditional approval where appropriate – allowing earlier access for particular groups of patients based on promising phase II data.

- Regulators, industry, academia and networks of clinicians and patient groups need to commit to a model of greater collaboration, transparency and risk sharing to steer treatments over the regulatory hurdles as fast as possible.

- The Accelerated Access Review was a welcome step towards earlier access to innovative treatments for NHS patients, but more needs to be done to provide faster access to a greater number of products. We welcome the establishment of a transformative designation that will provide accelerated regulatory approval, reimbursement, evaluation and adoption process for the most important new medicines and technologies. But only five products per year will benefit from this pathway, so we would like to see further changes that facilitate rapid approval of a greater number of innovative cancer drugs once their effectiveness becomes apparent.

- We need to test drugs in smarter, faster, more efficient trials to generate the required standard of evidence more quickly. We support greater use of innovative trial designs such as basket and umbrella trials, where patients are stratified by their specific tumour profile rather than simply their broad cancer type and stage, and adaptive trials, where the design can be modified according to how early participants have responded. Increasingly, regulators are approving drugs on the basis of phase II trials to speed up their development, and we believe intelligent trial design can play a vital role in reducing the time it takes for new treatments to reach patients.

- We welcome the fact that licensing agencies are assessing data from phase II trials. However, we need to build on this to speed up access to a wider

range of treatments. This could include provisional licensing of drugs based on strong surrogate, secondary endpoint or health-related quality of life data rather than simply a survival benefit, which is often difficult to demonstrate during a phase II trial, especially for trials involving small populations. This would allow more drugs to be brought to patients more quickly with more extensive survival data being provided later.

- We believe that continuous collection of data and evidence is important, including after the licensing of a treatment. Coordinated registries of patients should be maintained where treatments have been licensed on preliminary evidence. This would be of particular benefit to patients with rare diseases where small patient populations make it difficult to recruit large numbers of patients to clinical trials. Where industry benefits from earlier licensing, the onus should be on the companies involved to collect this data to allow the effectiveness of treatments to be monitored post-approval, informing their further use and possible future research.

- We believe the current regulatory requirements for validation of biomarkers can be prohibitive for early-stage trials and are not fit for purpose. If we are to speed up access to treatments, we need to employ greater use of precision biomarkers which can identify specific populations that benefit from a drug and help to generate clear evidence of benefit in these subgroups.

- It is especially important for the regulatory system to be flexible in supporting faster access to innovative treatments for rare cancers and cancer subtypes. Regulators need to accept greater uncertainty in assessing treatments for rare cancers because low patient numbers make it more difficult to demonstrate effectiveness. Adaptive trial designs and innovative statistical methods developed for small population sizes must be planned early in clinical development of treatments for rare cancers – and in coordination with regulators. Clinical trials for rare cancers should use specific groups of patients based on biomarker data to increase the chances of detecting meaningful endpoints from smaller groups.

- We support the repurposing of existing drugs to provide faster access to cancer drugs at lower cost when a drug has shown evidence of benefit in treating a cancer for which it is not currently licensed. Policy makers need to address the issues that prevent greater uptake of repurposed drugs such as a current lack of incentives to encourage off-patent repurposed drugs to be licensed for new indications and by providing a simple pathway for licensing evidence-based repurposed drugs without patents.

- We believe it is critical that efforts to promote wider use of off-label and repurposed drugs do not inadvertently undermine clinical trials. We believe that clinical trials are the most appropriate vehicle for testing new

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treatments, and measures to promote off-label prescribing should support participation in clinical trials.

- We need to ensure that the UK's drug regulatory system remains closely aligned with that of the European Union. If drug companies have to gain separate licences in the UK before they can market drugs here, UK patients could suffer significant delays in accessing the latest cancer treatments. The UK's drug regulatory system must continue to accept EMA approvals so that our patients do not miss out on the benefits of programmes such as Priority Medicines (PRIME).