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Some cancer drugs approved in Europe might not have sufficient evidence of survival benefits, says study

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Credit: Cancer Research UK

Most cancer drugs approved in Europe from 2009-2013 weren't backed by sufficient scientific evidence that they work, according to a new study.

At the time of approval, around a third of European approvals were backed up by robust evidence from clinical trials showing improved survival, and 1 in 10 approvals followed evidence that the treatment improved quality of life.

After further evidence was gathered around half of approvals had evidence of improving survival or quality of life.

Emma Greenwood, Cancer Research UK's director of policy, said the findings were useful, but might not reflect the drugs that are available in the UK as it has another layer of approval for drugs, and that the system has changed since the period under study.

"While the European Medicines Agency (EMA) grants market authorisations for new drugs, it is national bodies such as NICE that are responsible for approving drugs for routine use on the NHS," she said.

In the case of cancer drugs, in England some treatments not initially approved by NICE may be prescribed on the Cancer Drugs Fund (CDF) while more evidence is gathered.

The CDF was initially established in 2010, but didn't monitor the effectiveness of drugs once prescribed. It was reformed in 2016 to give patients access to treatments while their effectiveness is evaluated.

The study, published in the *BMJ* , looked at evidence for 48 drugs that the EMA approved for 68 uses in specific patient settings from 2009-2013.

It found that at the time of approval 24 of these 68 'indications' (35%) had evidence for increased survival, but for an average of less than 3 months. Only 7 of 68 (10%) had evidence that they improved quality of life.

Although small, survival benefits can accumulate as cancer drugs are often used in sequence, bringing greater benefit to patients towards the end of their life.

After approval, drugs were found to increase survival in 3 more settings, and to improve quality of life in 5 more settings.

Cancer drug development has changed with an increased focus on personalised treatments targeted to the biology of a patient's cancer, rather than for all patients with a particular disease.

This means that often there is a smaller number of patients being treated with these drugs and that clinical trials may include fewer patients.

Many new drugs, such as immunotherapies, are the first of their kind, raising additional questions for drug bodies looking to assess their effectiveness.

In an attempt to deal with these issues, programmes like the CDF aim to add to gold standard clinical trial data with evidence of how a treatment performs after being licenced.

"The study does highlight the importance of using real-world evidence from patients, on top of data from clinical trials, to build our understanding of how drugs work in a real-life setting," said Greenwood.

"We're already starting to see this happen through the CDF in England, where patients can access promising new drugs while more data is collected on their effectiveness. This type of evidence is becoming increasingly important as more innovative and targeted treatments are developed."

The study's authors said that patients can be harmed, money wasted, and

the aims of health services undermined when expensive drugs paid for by public money are approved without clinical evidence.

Dr Vinay Prasad, an expert in public health at Oregon Health & Science University in the US, and a critic of the current costs of drug development, said that rigorous testing and randomised clinical trials should be used to determine effectiveness.

"The expense and toxicity of cancer drugs means we have an obligation to expose patients to treatment only when they can reasonably expect an improvement in survival or quality of life," he said. The findings suggest "we may be falling far short of this important benchmark", he added.

More information: Courtney Davis et al. Availability of evidence of benefits on overall survival and quality of life of cancer drugs approved by European Medicines Agency: retrospective cohort study of drug approvals 2009-13, *BMJ* (2017). DOI: 10.1136/bmj.j4530

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