

NEWS

Seriously ill patients in UK will have access to unlicensed drugs

Nigel Hawkes

London

Patients in the United Kingdom are to be given access to some drugs before they have been formally licensed, in an effort to speed up access.

The early access scheme, which was first proposed in 2006 and has been under active discussion for the past two years,¹ will apply to life threatening and seriously debilitating conditions.

The first step is for the Medicines and Healthcare Products Regulatory Agency to designate a new drug as a “promising innovative medicine” (PIM) on the basis of early trial data and as long as the agency is satisfied the drug meets an unmet need. Drugs with PIM designation may, once enough data on quality, safety, and efficacy are gathered, be submitted for the second step: approval for use in the early access scheme.

The purpose is to leapfrog the licensing process, which can take a year or more. But the scheme is intended to process only a small number of drugs each year, reckoned to be between five and 12. Typically the drugs will be in or have been in phase 2 trials. Because the drugs will be unlicensed they will be used on a named patient basis, as unlicensed drugs already are, with legal obligations falling on the prescriber. Patients will need to be well informed and to give active consent.

The scheme’s purpose is twofold: to give patients who have conditions for which there is no effective treatment the earliest possible access to new drugs; and to encourage drug companies to see the UK as a good place to launch new drugs.

The idea was first proposed by David Cooksey in his review of the funding of UK health research, published in December 2006.

A plan was produced by November 2009 but languished until revived two years later. The scheme will not be unique—a similar scheme in France has operated for 15 years.

Jeremy Hunt, the health secretary for England, said, “Making Britain the best place in the world for science, research, and development is a central part of our long term economic plan. This groundbreaking scheme will provide cutting edge medicines earlier to give hope to patients and their families and save lives. And as part of our strategy for life sciences it will create more jobs and opportunities for people, helping secure a better future for our country.”

Harpal Kumar, chief executive of the charity Cancer Research UK, backed the scheme. “Time is of the essence for many cancer patients, particularly those with more advanced disease,” he said. “It can mean the difference between life and death. Therefore this scheme, which has at its heart the potential to bring promising new medicines to patients faster, is to be warmly welcomed.

“The scheme should also make it more attractive for life sciences companies to conduct their development activities in the UK, which will bring a multitude of benefits to the population.”

The scheme does not need new legislation and will be introduced in April.

1 Hawkes N. Speeding up access to new drugs. *BMJ* 2012;344:e999.

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