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NEWS

European scheme to develop drugs for diseases with no treatment launched

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The European Medicines Agency (EMA) has launched a scheme to speed up the development of new drugs that target diseases with no existing treatment.

Aimed at smaller companies and academic researchers, PRIME (PRIority MEdicines) aims to streamline the development process by offering early advice on clinical trial design. It said that much effort is wasted, especially by those less experienced in drug development, by poorly designed trials that do not address the issues needed for marketing authorisation.

Unlike the UK's Early Access to Medicines scheme, PRIME does not shorten the authorisation procedure. Under the UK scheme drugs may be given market access if they show sufficient promise before a phase 3 trial is undertaken. PRIME is closer to the US model, the Breakthrough Therapy Designation, which gives guidance and a chance for the drug to qualify for priority review.

"Our goal is to foster better planning of medicine development to help companies generate the high quality data we need to assess quality, safety, and efficacy of medicines," said Guido Rasi, the EMA's executive director. "Patients with no or insufficient treatments could then benefit from scientific progress and cutting edge medicines as soon as possible."

The most controversial aspect of PRIME is the advantage it gives to smaller companies and academics, who can apply earlier in the development process. They will be allowed to apply at proof of principle stage (before phase 2 trials) while larger companies will have to have conducted successful phase 2 trials before they can apply. This has attracted criticism from larger companies, who have argued that unmet need is not determined by whether a company is large or small.

The EMA has not backtracked and at a press conference to launch PRIME, Zaide Frias, head of regulatory affairs for the agency, said that small and medium sized companies needed more assistance.

Those who gained access to the scheme would get scientific advice, a meeting with experts to discuss the development programme, the early appointment of a rapporteur from the Committee for Medicinal Products for Human Use (CHMP, the committee that ultimately decides on market authorisation) and, for smaller companies or academics, the waiving of some fees. The EMA said that access to the scheme would make fundraising easier for start up companies.

Just how much difference it will make is not clear. Rasi said that two out of three trial designs had deficiencies that could have been avoided by earlier advice. Tomas Salmonson, who chairs CHMP, said that the EMA had had an accelerated access scheme for a long time, "but a month here or there isn't a big gain." The real gains were to be made by hastening the development phase, he said, adding "but how big those gains will be is a very hard question to answer."

He expected that most applications for the scheme would be turned down, since there were not that many that met the criteria. Frias said she expected around 100 applications a year, but Salmonson said that he thought there would be fewer.

There is some evidence that the US scheme has shortened development times, with drugs given Breakthrough Therapy designation being approved after an average development time of five years, compared with six or seven years for those outside the scheme.