See also p. 5 and 25

Drugs for rare diseases: baseless approvals

Rare diseases sometimes place affected families in tragic and desperate situations. These families are often passionately and vigorously defended by patient advocacy groups. Sometimes at the risk of endangering their cause.

A marketing authorisation to satisfy desperate parents. The US FDA has approved a new drug, *eteplirsen* (Exondys 51°), for the treatment of Duchenne muscular dystrophy, a disease that affects boys (1,2). It was authorised on the basis of three trials in 12 and 13 patients (3). Yet the FDA's panel of experts had considered that "*major flaws in both the design and conduct of the clinical trials using eteplirsen have made it impossible to use much of the resulting trial data as reliable evidence in regulatory decision-making*" (1-3). One FDA official also considered that Sarepta, the company that developed *eteplirsen*, had published misleading communications and fuelled unrealistic expectations of the benefits of the treatment (3). However, the director of the FDA's Center for Drug Evaluation and Research overruled the panel and approved the drug, because the flaws in the clinical evaluation should not be held "against the patients", among whom there was intense demand for access to this new drug (1-3). A demand fuelled by the company's communications.

Unjustifiable, unaffordable prices. When the news emerged that Exondys 51° had been approved, Sarepta's stock doubled within 24 hours (1). The treatment costs on average $300 000 per patient per year (2).

There is growing criticism of the high prices demanded for inadequately evaluated orphan drugs (4-6). For example, a former head of the French government's health product pricing committee (CEPS), has commented that "*orphan diseases account for a considerable proportion and weigh too heavily* [on the public purse]" (5). In the US, the private health insurance provider Anthem has decided not to cover the cost of Exondys 51° (6).

In practice, baseless marketing authorisations and astronomical prices for orphan drugs with uncertain clinical effects work against patients' interests.

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EDITORIAL