Orphan drugs: amendment needed

In 2000, the European Union adopted a regulation to encourage pharmaceutical companies to bring to the market orphan drugs, i.e. drugs to treat rare diseases (see "Gilteritinib and acute leukaemia" p. 117 and "Ciclosporin eye drops in severe vernal keratoconjunctivitis" p. 122 of this issue) (1). By 2006, the limitations of this regulation, and its abuse by some pharmaceutical companies, were already evident (2). A number of evaluations published in 2020 have confirmed these shortcomings (3).

The European regulation on orphan drugs was intended to encourage pharmaceutical companies to develop drugs for situations which they would otherwise have considered insufficiently profitable, and thereby give more patients access to treatment. But nothing in this regulation prevents pharmaceutical companies from setting the price of orphan drugs so high that they become inaccessible for many patients, while still bringing in huge profits for drug companies.

Back in 2006, the case of *imatinib* (Glivec°) showed that a drug could generate considerable sales revenue (almost $6 billion in total) by successively stacking up new indications, each benefiting from orphan drug status (2). By 2019, total revenues from Glivec° had exceeded €43 billion. And 10 other orphan drugs had each generated total revenues of over €10 billion (3).

In the face of growing criticism and the fact that many patients in Europe do not have access to orphan drugs, the European Commission conducted an audit of this policy (4). It welcomed the fact that 142 orphan drugs had been brought to the market since 2000, and that over 6 million patients had benefited from earlier access to these treatments as a result of the regulation. But it also acknowledged numerous limitations: only about 20 of the 131 orphan drugs still on the market would not have been marketed without this regulation; access to orphan drugs is greater in Europe's wealthiest countries; too many pharmaceutical companies focus on specific cancers and ignore the rarest diseases; and some companies take advantage of the regulation, mainly by stacking up a series of extremely narrow indications for the same drug. The Commission is therefore proposing the revision of certain rules in order to limit abuse and refocus the policy on neglected rare diseases.

Let's hope that the Commission will not delay in decisively amending the European orphan drug regulation, to ensure that it better serves patients' interests and that the public resources invested benefit public health rather than private interests.

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EDITORIAL

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