

Pricing of new drugs: deadlock

Is it possible for countries to provide access to new, very expensive drugs while balancing the books of their health insurance system? The pharmaceutical industry's pursuit of maximum profits is making it increasingly difficult to reconcile these competing pressures.

Since the 2000s, many pharmaceutical companies have switched their focus from "blockbusters" to "nichebusters". Blockbusters are drugs that generate very high revenues because they are sold in large quantities, whereas nichebusters are those sold at very high prices for a small number of patients, mainly those with rare diseases or cancer (1).

This business model is causing serious financial problems, even for wealthy countries. For example, in 2023, 2 of the 5 most costly drugs to France's health insurance system, dispensed in community pharmacies, were drugs for rare diseases: Vyndaqel® (*tafamidis*), dispensed to about 11 000 patients with transthyretin amyloidosis, and Kaftrio® (*ivacaftor + tezacaftor + elexacaftor*), dispensed to about 5000 patients with cystic fibrosis. These drugs cost between €50 000 and €100 000 per patient per year (2).

And with certain one-time gene therapies, each patient's treatment costs millions of euros. The 10 most expensive drugs in the US are gene therapy products, costing between \$2.2 million and \$4.3 million per patient per year (3). Only 2 of these drugs were marketed in France as of April 2025: Hemgenix® (*etranacogene dezaparvovec*) and Zolgensma® (*onasemnogene abeparvovec*), the published prices for which are similar to those quoted in the US (3).

These prices are very close to those that countries such as France are officially prepared to pay to save one human life, i.e. about €3 million (4). In other words, pharmaceutical companies take the fullest possible advantage of what the wealthiest countries are willing to pay.

This pricing strategy is making it increasingly difficult for large swathes of patients around the globe to access new treatments, which poses a problem when the drug concerned represents a real therapeutic advance. An international citizen movement has petitioned South Africa, Brazil, India and Ukraine not to recognise the patents on Kaftrio®, which is beyond the reach of patients in these countries (5).

The pharmaceutical company Bluebird Bio has abandoned plans to market Skysona® (*elivaldogene autotemcel*) in Europe, "because of a price-setting policy considered too hostile to advanced therapies" (our translation) (6). This is especially galling since this drug for early cerebral adrenoleukodystrophy, which retails for \$3 million in the US, originated through research conducted by French teams with funding from a French charity (7).

By demanding such high prices, the pharmaceutical industry is creating an ethical problem for all countries: providing access to excessively expensive new drugs that are little or no better than existing options can divert public funds away from their use in other, more cost-effective areas of health care (8).

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