EDITORIAL

Evaluating drugs for today's patients, without neglecting tomorrow's patients

The quantity and quality of information available about new drugs when they first enter the market has declined considerably in recent years, to the point where a group of drug evaluation specialists and healthcare professionals issued a warning about this situation in a French national newspaper in spring 2023 (1). At the same time, certain patients' organisations criticised France's National Authority for Health (HAS) for "deadly decisions" which they claim delay access to purportedly promising new drugs (2-4).

The fact is that patients' interests diverge in part when it comes to the evaluation of new drugs. It is in every patient's interests to have access to well evaluated drugs: drugs that have been shown to have a favourable harm-benefit balance, based on high-level evidence and meaningful clinical outcomes, in comparison with the best treatment options available. But patients' expectations differ, depending on their individual circumstances. For example, it is understandable that a patient with a serious disease for which no satisfactory treatment exists wants access to new drugs as rapidly as possible, even inadequately evaluated drugs, despite the considerable risk and uncertainty (5).

Drug regulatory agencies in the United States and Europe have made it possible for patients to access certain drugs at an increasingly early stage. This has increasingly encouraged pharmaceutical companies to seize the opportunity offered by these easy markets, with low barriers to entry and early profitability (6).

Drug regulators accept low-level clinical evidence when granting early marketing authorisation, and then, at the post-authorisation stage, fail to demand the quality of evidence required to complete the evaluation. This is a disservice to all patients, because treatment choices are destined to be made, for some time, on the basis of very fragile data (5). For example, by agreeing to authorise drugs for Alzheimer's disease on the basis of an effect on amyloid plaques, a surrogate endpoint that has not been shown to correlate with improved clinical outcomes, regulatory agencies are encouraging pharmaceutical companies to choose this relatively easy evaluation method, and to neglect other, potentially more promising, avenues of research (5).

Tight deadlines, to meet the needs of patients for whom no effective treatment options currently exist, rarely lead to robustly evaluated new drugs. In the interests of all of tomorrow's patients, pharmaceutical companies should be made to continue evaluating these drugs, authorised on the basis of fragile evidence, until their evaluation meets the usual standard, if only to prevent any further exposure to drugs that are ultimately shown to be more dangerous than beneficial.

Pharmaceutical research and development, overseen by drug regulatory agencies, must take into account both today's and tomorrow's patients.

Prescrire

References **1-** Autrant B et al. "Traitements contre le cancer: "Le bénéfice des nouveaux médicaments, évalué à court terme, est trop incertain"" *Le Monde* 19 May 2023: 1 page. **2-** AF3M "Halte aux décisions mortifères de la HAS: Mise en danger des malades du myélome multiple" 12 February 2023: 2 pages. **3-** Prescrire Editorial Staff "France's health technology assessment body: pushed to lower the bar!" *Prescrire Int* 2023; 32 (254): 305. **4-** "Dr Étienne Lengliné, de la Commission de la Transparence "Il y a actuellement beaucoup d'incertitudes dans l'évaluation des médicaments"" *Lymphom'Action* 2023; (50): 8-11. **5-** Lynch HF and Largent E "Considering tomorrow's patients in today's drug approvals" *BMJ* 2023; 381:e07500017: 4 pages. **6-** Prescrire Editorial Staff "Pharmaceutical research and development: a new system is needed" *Prescrire Int* 2017; 26 (182): 130-135.

Downloaded from english.prescrire.org on 27/08/2025
Copyright(c)Prescrire. For personal use only.