

Too short-term

Some diseases progress slowly, with the main complications occurring years or even decades after diagnosis.

It is in patients' interests that marketing authorisations for new drugs should be based on an evaluation conducted for a period approaching the duration of patients' exposure to the drug in the real-life setting. In addition to identifying any potential short-term effects, the drug's efficacy in treating the disease as well as adverse effects in the long term would be known. However, this approach is not often adopted: in general, evaluations are short-term or last only long enough to detect the slightest hint of effects that often remain highly dubious.

Three recent examples: *obeticholic acid* (Ocaliva®) evaluated for only one year in primary biliary cholangitis (to be covered in an upcoming issue); *canakinumab* (Ilaris®) in periodic fever syndromes (to be covered in an upcoming issue) and *mercaptopamine* eye drops (Cystadrops®) in cystinosis (to be covered in an upcoming issue), each evaluated for a few months. The limited nature of the evaluation, which fails to demonstrate what the drug actually offers beyond questionable extrapolations, leads to premature marketing authorisation.

Patients consequently take drugs with relatively well elucidated short-term effects, apart from those that only occur infrequently, but highly uncertain long-term effects. Over time, a drug's efficacy may prove marginal compared to its adverse effects as they become better known, and its harm-benefit balance will belatedly be considered unfavourable. Ultimately, it is patients who pay the price for this inadequate evaluation. The withdrawal of the marketing authorisation for *daclizumab* (Zinbryta®) is one such example (pp. 173-175).

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