Non-comparative trials for marketing authorisations: EMA consultation

Prescrire has contributed to a consultation on the use of non-comparative clinical trials to obtain marketing authorisation.

In September 2023, Prescrire submitted its response to a public consultation organised by the European Medicines Agency (EMA) on the use of non-comparative clinical trials as the main (“pivotal”) evidence of efficacy in marketing authorisation applications (1,2).

Prescrire considers that the reflection paper rightly highlighted the methodological weaknesses of non-comparative trials in evaluating the potential efficacy of a drug. It is because of these weaknesses that, with a few rare and substantiated exceptions, marketing authorisations should not be based on such trials. Prescrire felt it was regrettable that the EMA’s preparatory document:

- Does not clearly spell out what these trials can do (generate hypotheses) and what they cannot do (demonstrate a causal relationship between the treatment and the outcomes observed);
- And does not define, from the outset, the handful of exceptional situations in which the use of a non-comparative trial might be considered an acceptable basis for marketing authorisation.

Drawing on concrete examples, Prescrire expressed its concern about the fact that, despite the known weaknesses of non-comparative trials, the EMA is increasingly accepting them as the sole basis for marketing authorisations (2).

Drug shortages: Prescrire calls for transparency

In October 2023, ahead of the publication of a Communication from the European Commission on addressing medicine shortages in the European Union (EU), a joint letter was sent to the Commission by Prescrire, the European Public Health Alliance (EPHA) and the patient rights umbrella organisation France Assos Santé (1,2).

The Commission is in favour of introducing a “Voluntary Solidarity Mechanism” across EU member states to address drug shortages. The cosignatories of the letter emphasised that transparency about drug stocks will be needed if such a mechanism is to work. They urged the Commission to call for the introduction of requirements for manufacturers and wholesalers that provide information on stock levels to the EU’s national drug regulatory agencies.