

Selected references from Prescrire's literature search

- 1- Gyawali B et al. "Regulatory and clinical consequences of negative confirmatory trials of accelerated approval cancer drugs: retrospective observational study" *BMJ* 2021; 374: n1959: 8 pages.
- 2- Prescrire Editorial Staff "Exploratory trials, surrogate endpoints: not a robust basis for marketing authorisation" *Prescrire Int* 2018; 27 (193): 137-138.
- 3- CBIP "Critique des critères d'évaluation intermédiaires en oncologie" *Folia Pharmacother* 2022; 49 (7): 1-7.
- 4- Gyawali B et al. "Assessment of the clinical benefit of cancer drugs receiving accelerated approval" *JAMA Intern Med* 2019; 179 (7): 906-913.

## France's health technology assessment body: pushed to lower the bar!

● The HAS's new principles for rating drugs, designed to facilitate access to new treatments, allow lower-quality evaluation. This may not be in patients' best interests.

In early 2023, the "Transparency Committee", which is part of the National Authority for Health (HAS), France's health technology assessment body, published its new principles for rating the "clinical benefit" provided by new drugs, and their "clinical added value" (i.e. whether they represent a therapeutic advance over existing alternatives). These two ratings affect drug pricing and the level of reimbursement provided through the national health insurance system (1).

While reaffirming that randomised comparative clinical trials are "the prerequisite and the essential reference for the assessment of any medicinal product", the HAS now also considers it acceptable to (our translation) "incorporate less well consolidated data, provided they enable [drugs to be] compared to the treatments available" (1,2). Behind this decision to accept lower-quality evidence lies a request from the French government, pushing the HAS to adapt its methodology to new types of clinical trials, in particular those employed by pharmaceutical companies to bypass randomised comparative trials.

Under the HAS's new principles, it becomes acceptable to assess new drugs through indirect comparison alone, against data

obtained on historical controls or on groups of patients enrolled in other clinical trials or in cohort studies ("external" control groups) (1,2). The bar has been lowered.

Under the HAS's new principles, it is likely that fewer drugs will be rated as offering insufficient clinical benefit and no clinical added value, and that more drugs will be eligible for reimbursement in the community and approved for use in hospitals, despite weaknesses in their evaluation. This development fits with France's new "early access" scheme, in which the national health insurance system covers the cost of drugs in certain clinical situations before they have been assessed by the Transparency Committee (3). The new principles would help to avoid authorisations issued through this programme which are later assessed as offering insufficient clinical benefit, as occurred with the former temporary authorisation for use (ATU) programme, with the Transparency Committee subsequently recommending that reimbursement of at least one indication be revoked for one-quarter of the drugs granted an ATU between 2014 and 2021 (4).

The new principles were also undoubtedly developed in response to lobbying by the pharmaceutical industry, certain

patient groups and medical specialists (5-7).

Many new drugs are poorly evaluated. Although certain parties want ultra-rapid access to new, poorly evaluated drugs, they should not, in the process, strip clinical benefit and clinical added value of their meaning. These ratings provide useful information about the value of drugs, information that benefits a great many current and future patients. And society as a whole, when deciding how best to allocate the resources at its disposal.

©Prescrire

► Translated from *Rev Prescrire* August 2023  
Volume 43 N° 478 • Page 616

Selected references from Prescrire's literature search

- 1- HAS "Doctrine de la Commission de la Transparence" 15 February 2023 + Press release 17 February 2023: 38 pages.
- 2- Vanier A et al. "Rapid access to innovative medicinal products while ensuring relevant health technology assessment. Position of the French National Authority for Health" *BMJ Evid Based Med* 2023; online: 5 pages.
- 3- Prescrire Editorial Staff "Early and compassionate access to drugs in France" *Prescrire Int* 2023; 32 (248): 136-137.
- 4- Juillard-Condat B et al. "Articulation entre l'accès précoce et l'accès de droit commun aux médicaments: quel impact peut-on attendre de la réforme de 2021?" *Panorama de droit pharmaceutique*; 2023 (10): 9-27.
- 5- Leem "3 défis pour 2019: l'accès des patients à l'innovation, la reconquête industrielle, et l'amélioration de la réputation du secteur" 15 January 2019: 10 pages.
- 6- Association Française des Malades du Myélome Multiple "Mobilisons-nous contre la mise en danger de la vie des malades par la HAS" [www.af3m.org](http://www.af3m.org) accessed 11 April 2023: 2 pages.
- 7- APM "Plus de 150 oncologues interpellent Emmanuel Macron sur les difficultés d'accès aux nouveaux anticancéreux" *APMNews* 5 April 2023: 3 pages.