HOW CAN WE IMPROVE DRUG ASSESSMENT?

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The evolution of drug assessment is necessary in the face of increasing disruptive innovations and personalized medicine

Therapeutic innovation is booming, with a significant increase in the number of available treatments. However, introducing these innovations to the market depends on a thorough assessment that considers the level of risk for patients and financial aspects, as well as assessing the quality, effectiveness, and efficiency of the drugs. The emergence of disruptive innovations, especially personalized medicine tailored to individual patient characteristics, necessitates adapting the benefit/risk assessment based on different situations, populations, and diseases to allow the safest possible access to these treatments. In this regard, the assessment of drugs for rare diseases, with target populations of small sample sizes, has already started to evolve towards other types of studies beyond randomized trials (indirect comparisons).

Performance contracts: A lever to address the high costs of innovative drugs?

One-third of products do not reach the French market even though they are available at the European level, primarily due to reimbursement pricing. However, some treatments have a beneficial impact on both society and finances that goes well beyond the cost of making the drug available. Furthermore, the lack of on the effectiveness of certain innovative drugs with marketing authorization (AMM) can be a barrier, given their high price. Performance contracts help remove these barriers and facilitate access to these treatments by implementing a pay-for-performance system. But these contracts are not unanimously accepted because it is difficult, in some cases, to evaluate a product based on a single criterion: performance.

Early Access Authorization (AAP): Faster access to products remains exceptional

When the data for an innovative drug is insufficient for marketing authorization (AMM), the product can benefit from an early access authorization (AAP). In this case, a mutual agreement is reached between the manufacturer and the public authorities based on the anticipated positive effects of the drug. In line with the expectations of the industry, the 2021 Social Security Finance Law introduced clear eligibility criteria and reduced time for market access. However, the number of molecules reaching the French market through early access authorizations is far from the number of molecules validated in the official drug compendium (Pharmacopoeia). These procedures are therefore exceptional and limited to the most innovative products.

The improvement of medical benefit is sometimes hard to quantify for early access authorizations

In some cases of early access authorization (AAP), the lack of data on therapeutic progress implies an assessment of the improvement of medical benefit (ASMR) at level 5, meaning the absence of therapeutic progress. While about 80% of early access results in improvements of medical benefit provided at levels 1 to 4, confirming the presumed degree of drug innovation, some labs are hesitant to market their product due to an automatic classification at level 5. A pragmatic 2024 Social Security Finance Law (PLFSS) is therefore needed to address these unquantifiable improvements of medical benefit, to allow early access authorization to continue over time, with controls being put in place.

Delays in access to drugs are improving

The average time between the marketing authorization of a drug at the European level and actual access for patients in France is 500 days. The French National Authority for Health (HAS) has made significant progress in terms of delays, thanks to a major overhaul of the process. Although a drug can be marketed as soon as it receives its marketing authorization, its reimbursement depends on the publication in the French Official Journal, indicating its price and reimbursement rate, the publication delay of which can be easily reduced.

It is necessary to increasingly involve patients in drug assessments

The assessment process does not give enough importance to patients. Their input should not be limited only to urgent cases of drug requests but should be included in the assessment process. Patient associations can have significant weight in the decision-making process for drug authorization as they provide a critical perspective on products.