New drugs: the right to know

According to its officials, the role of the European Medicines Agency (EMA) is to ensure that patients have timely access "to medicines that are safe, effective and of suitable quality, as well as the information needed to use those medicines and make informed choices about their treatment" (1). Does the EMA really fulfil these missions?

Since the second decade of the 21st century, more and more publications have been critical of marketing authorisations (MAs), both in the United States and Europe. These publications show that a very significant proportion of drugs are introduced to the market in the absence of any proof of tangible clinical benefit for patients (2-5).

For example, in 2019, a study showed that more than half of all drugs entering the German market had no demonstrated added benefit over standard care (2). Another study showed that the majority of drugs authorised in Europe between 2011 and 2018 through the accelerated MA procedure were evaluated on the basis of surrogate endpoints without any correlation with clinical outcomes (3). A study of cancer drugs approved by the EMA between 2014 and 2016 showed that half of the randomised clinical trials supporting the MAs were probably biased in their design, their conduct or the analysis of their results (4).

According to EMA's medical director, patients have to accept a significant level of uncertainty with regard to new drugs (6). EMA officials recognise that "more emphasis should be placed on contextualizing the effect of new medicines and on being more explicit about negative, neutral or positive added benefit where possible in relevant patient subgroups" (7). These officials had already recognised in 2018 that the added therapeutic benefit of a new drug could be negative (8).

Of course, uncertainties exist at the time of marketing authorisation, but what is the EMA doing to explain these uncertainties to patients? What is the EMA doing to make healthcare professionals and patients aware that new drugs authorised in Europe can have a "negative" added therapeutic value, i.e. represent a step backwards?

We look forward to the EMA explaining and providing details in drug information documents, particularly in the summary of product characteristics and patient information leaflets, concerning the weakness of the clinical evidence supporting the marketing authorisation, and the magnitude of the resulting uncertainties regarding the real therapeutic benefit of drugs. This would enable healthcare professionals and patients to make more informed choices, just as the EMA is claiming in its mission statement.

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Volume 40 N° 435 • Page 56

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