Adaptive pathways: EMA still leaves open questions unanswered

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At the beginning of August, the European Medicines Agency (EMA) published a report on a pilot project of a new accelerated path for drug approval (adaptive pathways). In this approval procedure, for a highly restricted population drugs are to be launched on the market faster based on less evidence. Further data on effectiveness, safety, and benefit for broader use are only to be generated after drug approval within everyday health care - as so-called real world data.

The German Institute for Quality and Efficiency in Health Care (IQWiG) again sees its concerns about adaptive pathways confirmed by the EMA report. This is because evidently neither industry nor EMA has a concept as to how real world data can be used after drug approval to allow drawing reliable conclusions on benefit and harm.

Scientists and consumer advocates criticize concept

The concept has been criticized internationally for some time now: For instance, scientists not only question the assumptions underpinning the concept, but also point to difficulties with its implementation. The European Consumer Organization (BEUC) see risks for patients and criticizes the lack of transparency in the development and implementation of the concept. On several occasions, IQWiG too has critically discussed the adaptive pathways concept, most recently at its Autumn Symposium in November 2015.

Detailed information is lacking

EMA's final report on the pilot project, which started in 2014, was expected all the more urgently. However, the report now published fails to provide detailed information on the 7 test runs selected within the pilot project, the development programmes planned for these pilot cases, or the methods to be applied.

EMA justifies this information gap by referring to the confidential nature of the consultations and the trade and business secrets of the companies involved. However, in view of the importance of the pilot project for drug development and the potential consequences of the considerable changes in approval procedures for patients, concealment of the content and results of the discussions seems unacceptable.

Limitations of real world data become clear

But even without detailed information, the report allows conclusions on a key component of adaptive pathways, namely the use of real world data after drug approval. The limitations of such data, which became clear at the IQWiG Autumn Symposium, are confirmed by EMA's pilot project.

For instance, EMA draws an extremely sobering conclusion here, stating that "The majority of the plans were vague in terms of the purpose of collection of real world data to supplement RCTs, and on the practical elements for implementation there was insufficient detail in the submitted proposals to explore the refinement of the safety profile, and even less about to what extent efficacy could be confirmed or augmented in the post-authorisation phase. A critical discussion on the quality, potential for bias, and reliability of the data acquired in the post authorisation setting, and their suitability for regulatory and HTA purpose, was lacking."

EMA does not make its own proposals

If EMA views industry's proposals to be so meagre, one would expect that the Agency would make its own proposals as to how real world data could be used after drug approval - but one searches in vain for such proposals in their report. However, generating real world data after drug approval and using them in a way that allows robust conclusions on benefit and harm is a key component of the adaptive pathways concept. If this is still lacking, then it would be high time to pause for a moment and rethink the whole concept, instead of considering more drugs in the consultations on adaptive pathways, as planned by EMA.

Provided by Institute for Quality and Efficiency in Health Care

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