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Press release

Adaptive pathways: key learnings and next steps

EMA publishes report on pilot project and will organise workshop in December to further explore concept

The European Medicines Agency (EMA) has published a final report on the experience gained during its pilot project on adaptive pathways, a product development concept for medicines that address patients' unmet medical needs.

The pilot project, which has now ended, showed that adaptive pathways can bring multiple stakeholders together – regulators, health technology assessment (HTA) bodies, healthcare professionals and patients – to agree on a prospective plan to generate data on a medicine across its lifespan in areas of unmet medical need. Adaptive pathways can support medicine development in therapeutic areas where evidence generation is challenging, such as infectious diseases, Alzheimer's disease, degenerative diseases, and rare cancers.

Adaptive pathways can be defined as a planned, progressive approach to bringing a medicine to patients. It is not a new route of marketing authorisation; it makes use of existing regulatory tools. Under this approach, the medicine will first be authorised in a small patient population that is likely to benefit most from the medicine. Then, additional evidence is gathered over time resulting in progressive licensing adaptations to extend or restrict the previously authorised indications of the medicine.

In March 2014, EMA launched a pilot project to explore the practical implications of the adaptive pathways concept with medicines already under development. EMA invited companies to submit ongoing medicine development programmes which fulfil the characteristics of adaptive pathways: a staggered approval from very small, restricted patient populations to increasingly wider populations; a binding plan of post-licensing evidence gathering; and involvement of key stakeholders in the process.

During the pilot, EMA received 62 applications, 18 of which were selected for in-depth, face-to-face meetings with the participation of other stakeholders. At the end of the pilot, six of these applications had progressed to receive formal parallel advice by EMA and HTA bodies and one to benefit from simple scientific advice. The majority of the proposals received were considered not suitable for adaptive pathways, and the companies were advised to pursue traditional development routes.

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Adaptive pathways is still a developing concept which will be refined as more medicines are considered for this approach.

The pilot helped to identify a number of aspects for further reflection. These include the need for increased involvement of patients to assist in the selection of candidates for adaptive pathways, the definition of methodologically-sound strategies of real-world evidence collection to support the assessment of both efficacy and effectiveness and the potential involvement of payers - Member States' organisations responsible for decision on pricing and reimbursement – to provide input on pricing strategies.

EMA will further explore adaptive pathways in the context of its parallel advice with HTA bodies, which provides a framework to include additional stakeholders (e.g., patients and, if relevant, payers). Medicine developers who are interested in following the adaptive pathways approach should submit a proposal to EMA. An updated guidance document published today outlines the steps to follow.

To gather the views and proposals from its stakeholders on the adaptive pathways approach, the Agency will organise a workshop on 8 December 2016. Further information on how to register will be published on the EMA website in due course.

Adaptive pathways: a life-span approach to learning

Adaptive pathways makes use of existing approval tools, in particular conditional marketing authorisation which has been in operation in the European Union since 2006. It also builds on the experience gained with strengthened post-marketing monitoring tools introduced by the 2012 pharmacovigilance legislation.

This concept of medicine development and data gathering is not meant to apply to all medicines, but only to medicines that are likely to address an unmet medical need. The medicine development also needs to meet the characteristics of adaptive pathways.

As for any medicine, a marketing authorisation will only be granted if the balance of benefits and risks for a defined patient population is found to be positive; the same principles and legal tools apply as for any other new medicine.

A key aspect of adaptive pathways is the involvement of all relevant decision makers across the lifespan of a medicine, including those who decide about patient access in the Member States. Dialogue between these stakeholders is essential to help determine which medicines could be appropriate for adaptive (iterative) development, to jointly agree a data generation plan to meet the needs of regulators and HTA bodies, and to ensure the use of the medicines is well monitored and managed.

It is particularly important that all involved stakeholders agree upfront on a plan of post-licensing knowledge generation for a medicine, before it is authorised, and that the marketing authorisation holder commits to carrying out this plan. Once a marketing authorisation has been granted, the post-authorisation plan becomes a legally binding regulatory obligation.

Cooperation between stakeholders and a strong pharmacovigilance system are the basis for a systematic monitoring of the safety and the overall performance of a medicine in clinical practice; these are the two key elements underpinning the adaptive pathways concept.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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