Clinical trials: Two arms are better than one

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The German Institute for Quality and Efficiency in Health Care (IQWiG) has responded critically to a reflection paper by the European Medicines Agency (EMA) on the approval of new drugs based on single-arm studies.

The EMA correctly points out that studies without a control arm are subject to bias and that, in general, it is hardly possible to estimate causal effects from them. However, it does not provide clear criteria for limiting drug approval based on such studies to extremely rare exceptional cases.

The FDA shows how to do it

There is also no recommendation on external controls—in contrast to guidance published in February by the US Food and Drug Administration (FDA).

Beate Wieseler, Head of IQWiG's Drug Assessment Department notes: "The FDA clearly states that the likelihood of demonstrating the effectiveness of a drug with an external control is low, and strongly recommends a study design with an internal control—also for rare diseases."

"In addition, the FDA designates specific situations where externally controlled studies are generally not suitable, for example when the natural history of the disease is not well known or the disease course is variable. The EMA should include these points in its reflection paper."

Accelerated approval is not an end in itself

The situation is well known: in rare cases, single-arm studies can demonstrate the safety and efficacy of a new drug well enough to gain regulatory approval. But when it comes to its actual use in a health care system, the drug needs to be compared with existing treatment options—and this should happen as quickly as possible.

Beate Wieseler explains, "By publicly reflecting on the opportunities and

limitations of single-arm studies for approval, without describing consequences of their drawbacks, the EMA is doing a disservice to both drug companies and patients. Drug development is efficient when new drugs can be used immediately and appropriately in health care."

"To achieve this, studies should be conducted from the outset that are suitable both for approval and for integration into the health care landscape through health technology assessment (HTA). This is not about accelerating market access for new drugs per se, but about accelerating their evidence-based introduction into the health care system—for the benefit of patients."

More information: Full comment: www.iqwig.de/en/presse/iqwig-c… ents/2023-09-21.html

Provided by Institute for Quality and Efficiency in Health Care

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