Early benefit assessment increases transparency for study data

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Four years have passed since the introduction of the German Act on the Reform of the Market for Medicinal Products (AMNOG). AMNOG was primarily aimed at containing the increasing drug expenditure of the statutory health insurance funds. However, the early benefit assessment of new drugs as stipulated by AMNOG also reveals so far unpublished information from clinical study reports. Researchers from the German Institute for Quality and Efficiency in Health Care (IQWiG) have further examined this issue in an article published in the *British Medical Journal*.

Their conclusion: Company dossiers and dossier assessments performed by IQWiG, which are published during the early benefit assessment of new drugs, contain considerably more information than other publicly available documents on clinical studies - especially with regard to patient-relevant outcomes in approved subpopulations. The early benefit assessment of drugs may also be suitable as a model for more transparency in other countries or in other areas, such as the assessment of the benefit of non-drug interventions

Documents analysed for 22 studies

The IQWiG researchers examined a total of 15 dossiers containing 22 clinical studies, as well as the corresponding dossier assessments performed by IQWiG. They compared these documents with the conventional documents on the above clinical studies, that is, journal publications, entries in study registries, and so-called European public

assessment reports (EPARs), in which the European Medicines Agency (EMA) presents the evidence on new drugs.

They evaluated which proportion of all of the information on the methods and results of the studies were completely reported in each type of document. This evaluation concerned the approved patient population, that is, those patients who had received the new drug as stipulated in the summary of product characteristics. Depending on whether approval had been granted for all patient groups investigated in the studies submitted during the drug approval process, or only for some of these groups, either the information on the total study population was analysed, or only that on the approved subpopulation. The completeness of information in the conventional documents was then compared with the AMNOG documents, that is, the company dossiers and IQWiG's dossier assessments.

More knowledge on approved subpopulations

Whereas only 52% of study results were completely reported in the conventional documents in those cases where the total study population was analysed, the corresponding rate in the AMNOG documents was 89%. The difference was even greater in the approved subpopulations. For patient-relevant outcomes, the rate was only 5% in the conventional documents versus 70% in the AMNOG documents.

Beate Wieseler, Head of IQWiG's Drug Assessment Department, explains "To apply the vocabulary from our dossier assessments to AMNOG itself, and not to the drug under assessment: There is an indication of a major added benefit. The early benefit assessment reveals a large amount of additional information from clinical studies: This is beneficial for research, physicians, and patients, and can thus improve health care."

European harmonization must not endanger progress

In their article in the *British Medical Journal* the authors therefore propose the implementation of similar legal regulations in other countries. However, in their opinion, more can also be achieved in Germany. For instance, it would be easier to determine the benefit of non-drug interventions if, also in this area, complete study reports had to be submitted to health technology assessment (HTA) agencies and the relevant data from these reports had to be published.

"The fact that data and documents on new drugs today do not disappear in some drawer is not owed to voluntary self-commitments of pharmaceutical companies, but to the law", says the Institute's Director Jürgen Windeler. "AMNOG also shows that clinical study reports can be nearly fully published without damaging pharmaceutical companies."

Now the transparency level in Germany should be maintained. So-called "joint assessments" are currently being discussed in Europe, that is, benefit assessments conducted conjointly by several HTA agencies. For this purpose, common assessment methods must be agreed upon. But Windeler warns: "This harmonization must not be an adjustment downwards. On the contrary; the transparency achieved by AMNOG should also become the standard in other European countries."

More information: www.bmj.com/content/350/bmj.h796

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