Extremely expensive medicines endanger solidarity-based health care system

The increasing trend towards high-priced medicines threatens the financial sustainability of health care systems. In a study, the Austrian Institute for Health Technology Assessment (AIHTA) has now developed - as an example - a good practice model with which the risks can be shared equally between the public sector and pharmaceutical companies.

It is considered the most expensive drug in the world, costing around 1.7 million euros per injection. Its name is Zolgensma. It is intended to treat children affected by spinal muscular atrophy (SMA) - a rare genetic defect in which the nerve cells in the spinal cord that control the muscles gradually die off. In Austria, about nine children are born with SMA every year. If Zolgensma is administered in time, the muscle atrophy can be stopped and - so the hope - a largely normal life can be made possible. In contrast to other therapies, only one injection is necessary. The alternative product Spinraza, for example, has to be injected every two to four months during the first year, and a ten-year therapy costs about 2.6 million euros. Combination therapies of Zolgensma and Spinraza are also already being applied.

"In recent years, it has become apparent that the market entry of high-priced drugs has increased, with between two and four additional therapies being approved every year. This trend threatens the financial sustainability of the health system," says Claudia Wild, head of the Austrian Institute for Health Technology Assessment (AIHTA). Most of them are gene or cell therapies and other therapies for rare diseases, so-called "orphan drugs". However, it is difficult for hospitals and health insurances to assess whether the therapies have the hoped-for effect, because "at the time of approval, there is usually little data on the actual medium- to long-term benefit of these medicines. The approval is often based on small, mostly single-arm studies in which there is only an observation group and no control group," Claudia Wild explains. This also means that the payers have to make their reimbursement decisions under great uncertainties and under public pressure from the patients concerned, who naturally place great hope in the new therapies.

Sharing risk

One way of sharing the risk fairly between the public sector and the manufacturing companies is through so-called "Outcome-Based Managed Entry Agreements" (OBMEAs). Through such agreements, therapies are financed by the public sector under clearly defined conditions. In a study, the AIHTA has now looked at what international experience already exists with OBMEAs. A systematic literature review and eleven interviews with 15 experts from eight different countries generated a good practice model that is to serve as a model for the implementation of OBMEAs in Austria.

The first step is to **initiate** an OBMEA. Here it is clarified for which therapies clearly defined agreements are to be made. The concrete purpose is also determined in this phase. "It is important to determine which uncertainties exist and which data are needed to eliminate these uncertainties," Claudia Wild explains. It must be clear from the outset what clinical results must be achieved for cost coverage by the health insurances.

The second step is the **study design**: which patients are to be included in the study, which relevant endpoints are to be collected. It must be clarified what benefit is expected from the therapy. This also means that it must be determined at what point the therapy is discontinued. The data are collected in registers, which are ideally international. "The public sector should take over the financing and have data sovereignty over these registers," emphasises Claudia Wild.

The third phase of an OBEMA is **evidence generation**. The agreed data are collected according to a predefined schedule and the data quality and validity are checked regularly. Reimbursement is only made if the data documentation is complete.

Finally, the OBMEA is **re-evaluated** and clinicians and patients are involved in the interpretation of the results. In addition, a decision will be made as to whether reimbursement will continue under the existing arrangements or under modified conditions.

Evidence-based decisions

The example of Spinraza and Zolgensma is also a good illustration of how important OBMEAs will be in the future. The therapies have been on the market in Europe since May 2017 (Spinraza) and May 2020 (Zolgensma), so data on the effectiveness of the therapies have been collected for several years. An - yet unpublished - systematic review of 22 studies - mainly on Spinraza - showed that not all children respond equally to the therapy and start to sit or to hold their head. For those children who needed invasive or non-invasive ventilation or tube feeding support, the therapy had no effect on these two vital functions.

The Austrian provider institutions, hospitals and health insurances, have already reacted cautiously and only pay when children reach a certain "milestone". "However, these contracts are not made public. We don't know what the defined milestones are and how many children reach these milestones," Claudia Wild criticises. According to the expert, what is needed are completely transparent agreements that are also communicated to patients and relatives. "Last but not least, the pricing of these expensive therapies by the pharmaceutical industry should be questioned, especially since the basic research of the therapeutic approaches has already been paid for by public research funding agencies," emphasises Claudia Wild.

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