National Health Care Institute

Evaluations Of Cost-Utility Analyses Of Pharmaceuticals Receiving Market Authorization Based On Single-Arm Trials In The Netherlands

Objectives

In recent years, multiple pharmaceuticals have been submitted to health technology assessment (HTA) bodies based on data from single-arm trials (SATs). Since this trial design lacks a comparator arm, this places additional challenges on analyzing the cost-effectiveness of these pharmaceuticals. This study aims to provide an overview of previous economic evaluations by Zorginstituut Nederland (ZIN) assessing pharmaceuticals based on SATs.

Methods

ZIN assessments of pharmaceuticals receiving market authorization by the European Medicines Agency (EMA) based on SATs in the period of 2018-2020 were investigated. If a cost-utility analysis (CUA) was performed, then the approach used to create a comparator arm, the main critique points from the scientific advisory board (WAR), and the final conclusion of the economic assessment were obtained.

Results

Of the 22 included EMA-approved pharmaceuticals: 12 were assessed by ZIN, whilst only 3 of 12 included a CUA. The reasons why a CUA was not conducted were: joint EUnetHTA assessment (N=1), does not comply with established medical science and medical practice (N=3), budget impact below €10 million (N=1), equal therapeutic value claim by manufacturer (N=4). The methods to obtain a comparator arm in the 3 economic assessments were: literature-based cohort (N=2) and intracohort control (N=1). The main critique of the WAR in all 3 assessments was the high level of uncertainty of the indirect comparison used to determine the relative effectiveness. This resulted in no incremental cost-effectiveness ratio being presented in 2 of the 3 assessments. All 3 assessments concluded that the pharmaceutical was not cost-effective.

Conclusions

SAT-based EMA approvals put significant challenges on HTA bodies to determine the cost-effectiveness of a pharmaceutical. This is mainly caused by the high uncertainty of an indirect comparison to determine relative effectiveness. Future research should explore more robust methodologies to obtain a comparator arm.

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