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AOTMiT recommendations in specific therapeutic areas compared to other HTA agencies

STRESZCZENIE W JĘZYKU ANGIELSKIM

Introduction: In 2005, Poland, following the example of numerous other developed countries, incorporated the *health technology assessment* (HTA) system into the drug reimbursement process. The Agency for Health Technology Assessment was established. In 2009, it received statutory authorization and a new name – the Agency for Health Technology Assessment and Tariff System (pl.: *Agencja Oceny Technologii Medycznych i Taryfikacji* AOTMiT). AOTMiT is an independent entity that collects data, develops analyses, and issues recommendations regarding the justification for public funding of drugs, medical devices, foods for special medical purposes, and healthcare services. AOTMiT's analytical data and recommendations support the Ministry of Health in making reimbursement decisions. Various management models are applied in HTA agencies across Europe and the world, and the recommendations issued can be positive, negative, or conditional. Therefore, the following research questions were formulated: Are the differences in recommendations issued by HTA agencies in specific therapeutic areas for the same drugs significant or negligible? What are the reasons behind differences in recommendations issued by HTA agencies in specific therapeutic areas – are they driven by varying interpretations and assessments of clinical data, or by economic considerations? Answers to these questions were sought through a comprehensive analysis of the recommendations of both European and non-European HTA agencies.

Objectives of the Study: The primary objective of the doctoral dissertation was to analyze AOTMiT's recommendations in comparison with other HTA agencies in various therapeutic areas. Additional objectives included the analysis and comparison of reimbursement systems in the studied countries, with a particular focus on HTA agencies, as well as an analysis of the time elapsed between drug registration and the issuance of reimbursement recommendations by HTA agencies in various therapeutic areas.

Material and Methods: In the first stage of the doctoral dissertation, a review of national and international literature was conducted to accurately characterize the reimbursement systems operating in Poland, as well as in England, Scotland, Wales, Ireland, France, the Netherlands, Germany, Sweden, Norway, Canada, Australia, and New Zealand. Particular focus was placed on the role of HTA agencies in the reimbursement decision-making process.

In the second stage, a structured database was created to extract data from reimbursement recommendations issued by the analyzed HTA agencies for new molecules or new indications registered by the European Medicines Agency between 2014 and 2019. HTA agencies for comparison were selected based on their experience in health technology assessment and their reference value for AOTMiT in terms of developing reimbursement recommendations.

Once the necessary data were gathered, the consistency of the analyzed HTA agencies regarding the issued recommendations was assessed. The analysis covered two key areas:

1. The analysis of quotation aimed to determine how often different agencies referenced specific criteria – clinical benefits, safety profile, cost-effectiveness, and budgetary impact – in justifying their recommendations.
2. The analysis of the appraisal examined the impact of the aforementioned criteria on the nature of the issued recommendations (positive/negative).

Finally, in the third stage, which supplemented the second stage of research, an analysis was conducted on the time elapsed between the registration of new drugs or indications and the publication of recommendations by HTA agencies in specific therapeutic areas. The aim was to identify potential disparities between Poland and other countries.

Results: All analyzed agencies conduct national assessments, and some also prepare regional assessments. The agencies are independent entities, in selected countries (in England, Australia, Norway, and Scotland) make reimbursement decisions, leaving financing issues to other public institutions. In most countries, the criteria for HTA evaluation include clinical efficacy, safety profile, medical need, and degree of innovation. Except for French and German agencies, HTA entities also consider the budgetary impact and cost-effectiveness. The main differentiation among the analyzed agencies lies in the adopted cost-effectiveness threshold, which remains officially undefined in half of the studied countries.

The analysis covered recommendations issued by 12 HTA agencies – a total of 2,496 reimbursement recommendations for 464 medicinal products and 525 indications.

In almost all countries – except for Wales and Ireland – the proportion of positive recommendations was higher than that of negative ones. The proportion was only slightly higher in Poland, Australia, and Germany. Therapeutic areas were identified where positive recommendations were published significantly more frequently than in others. The degree of agreement regarding the type of reimbursement recommendations for specific indications in paired comparisons of HTA agencies was moderate.

Comparisons of pairs of agencies concerning the use of clinical efficacy in formulating recommendations revealed similarities between the Polish agency and six others: English, Scottish, Irish, French, German, and Canadian.

Regarding references to cost-effectiveness in recommendations, Poland demonstrated moderate similarity with England, Scotland, Ireland, Australia, and Canada. However, in terms of cost-effectiveness valuation, the Polish agency was similar only to the Irish and Canadian agencies.

Similarly to cost-effectiveness, in Poland, Australia, and Scotland, demonstrating a positive budgetary impact facilitated the issuance of positive recommendations. However, while in Scotland, such references were more often used to justify positive recommendations, in Poland and Australia, they were more commonly linked to negative ones.

Differences were observed between countries concerning the time from drug registration to the publication of recommendations. In Wales, Germany, Australia, France, Scotland, and Norway, the average period did not exceed one year. When considering the median, the time between registration and issuing recommendations exceeded one year only in Ireland, New Zealand, and Poland.

Conclusions: Although the subject and scope of HTA is precisely defined, the location of the relevant HTA agencies and the impact of their recommendations on the reimbursement system is different. The Polish reimbursement recommendation system shows some similarities to the systems used in Canada and New Zealand, particularly in the nature of issued recommendations (positive/negative), more so than to systems in other European countries. Regarding the justification pattern, the greatest similarity was noted between AOTMiT and the Irish agency.

When issuing final recommendations, the Polish agency takes into account four key aspects: clinical efficacy, safety profile, cost-effectiveness, and budgetary impact. Compared to other countries, the justification pattern in Poland differs primarily in the analysis of budgetary impact and, to a lesser extent, cost-effectiveness.

The waiting time from drug registration to the publication of recommendations by AOTMiT is among the longest of the analyzed agencies. However, since 2017, this period has been systematically shortening.