CEE: Brief overview of orphan drug reimbursement and selected clinical characteristics

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OBJECTIVES

Policymakers in different countries consider various aspects of orphan drug reimbursement. In Central and Eastern European (CEE) countries, some common characteristics can be seen when it comes to strategies for a drug policy. We decided to investigate which aspects of the clinical data available for orphan drugs influence drug reimbursement in selected CEE countries.

METHODS

Data was collected within a questionnaire study in a group of experts from nine CEE countries between September 2021 and January 2022; it was categorized, and statistical analysis was performed. An online survey was conducted for: Bulgaria, Croatia, Czechia, Estonia, Hungary, Lithuania, Poland, Romania, and Slovakia. Safety and efficacy (effectiveness) of orphan drugs was considered in a reimbursement process in all analysed countries

RESULTS

At the time of the study (September 2021), 125 drugs with orphan designation authorised by the European Medicines Agency (EMA) were revealed. The country with the highest number of reimbursed orphan drugs was Hungary (62; 49.6%), followed closely by the Czech Republic (61; 48.8%), while the least number of reimbursed drugs was observed in Estonia (22; 17.7%) – a mean number of reimbursed drugs was 40.3 ($p<0.001$). The impact of orphan drug safety and effectiveness on reimbursement decisions was rated according to experts claims as strong in 56%, moderate in 22%, and low in 22% of countries **(Figure 1)**. The acceptable safety profile of an orphan drug was a mandatory reimbursement criterion in 44% of countries, but in 56% of countries no separate safety evaluation was required **(Figure 2)**. However, the evaluation of the efficacy of orphan drugs was mandatory in the reimbursement process among 78% of countries, partially mandatory in 11%, and not required in 11% **(Figure 3)**. Additional other clinical aspects (eg. highly innovative medicinal product status) influenced reimbursement decision in 44% of countries.

KEY FINDINGS: The chances of reimbursement for orphan drugs in CEE countries were reduced by 38% (OR = 0.62) in countries where a level of impact of safety and effectiveness was rated strong compared to countries with low rating ($p<0.05$).

Figure 1. The impact of orphan drug safety and effectiveness on reimbursement

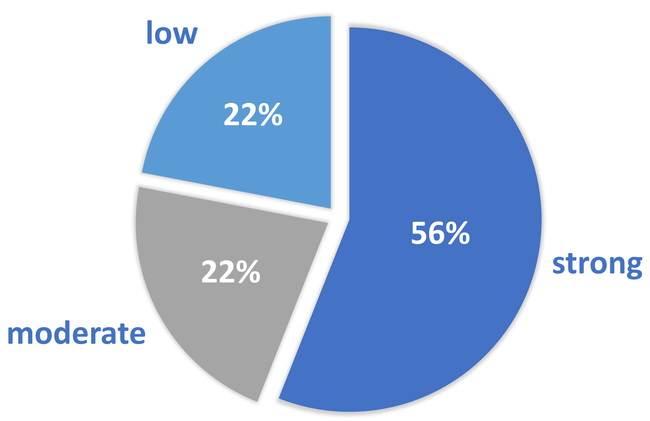


Figure 2. The acceptable safety profile of orphan drugs as reimbursement criterion

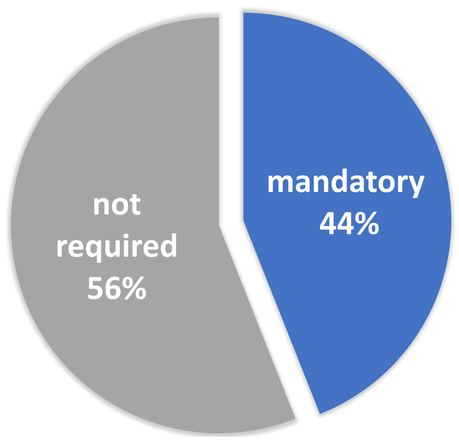
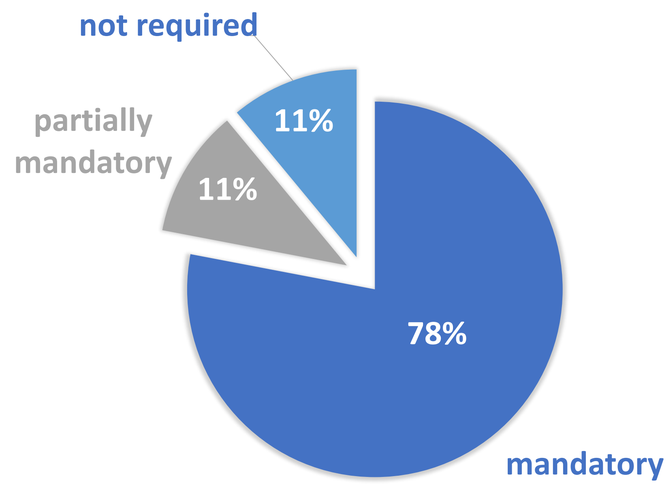


Figure 3. The evaluation of the efficacy of orphan drugs in the reimbursement process



CONCLUSION

Preliminary findings indicate differences between countries in orphan drug reimbursement policies in the area of clinical aspects (safety and effectiveness). This reveals the complexity and difficulty in evaluating orphan drugs. The poster is part of a larger study, the detailed results of which will be available in a forthcoming publication.

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Find me!

Szczepan Jakubowski

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