Managing Uncertainty for the Orphans: a Belgian Perspective on Managed Entry Agreements for Orphan Drugs

Alessandra Blonda[1], Isabelle Huys[1], Yvonne Denier[2], Steven Simoens[1]*

[1] Department of Pharmaceutical and Pharmacological Sciences, KU Leuven, Belgium; [2] Department of Public Health and Primary Care, KU Leuven, Belgium
* contact: alessandra.blonda@kuleuven.be

BACKGROUND

- Managed entry agreements (MEAs) are increasingly used as a tool to address the financial and clinical uncertainties that often remain at the time of orphan drug reimbursement.
- MEAs allow a temporary reimbursement provided that real-world evidence (RWE) is collected to clarify these uncertainties
- In addition, they may include price-discounts or measures for risk-sharing, such as pay-for-performance measures, linking price under agreement to specific outcomes
- Meanwhile, the confidentiality concerning the specific measures included in the MEA and the 'real' price under agreement of an orphan drug is often criticized, leaving stakeholders wondering whether the MEA succeeded at solving the uncertainties at the end of the contract term, and whether the orphan drug met the conditions as set forth by the MEA.

AIM

The aim of our study was to analyze and describe the MEAs for orphan drugs in Belgium. In particular, the study aimed to identify the uncertainties that remained at the time of their reimbursement, and describe the RWE that was collected for their reassessment.

METHODS

- We analyzed the public parts of the Belgian MEAs for all currently reimbursed orphan drugs in Belgium
- We extracted several characteristics of these MEAs, such as the type of MEA, the uncertainties included and the RWE collected in response to these uncertainties
- Uncertainties were categorized according to the nomenclature as provided by the Federal Knowledge Center for Healthcare (KCE) in Belgium.

RESULTS

31 orphan drugs representing 84 MEAs:

6 of 31 Orphan drugs were reassessed

- 1 orphan drug granted definitive reimbursement in all indications
- 1 orphan drug granted reimbursement in one of two indications
- 4 remaining orphan drugs were denied definitive reimbursement

25 of 31 Orphan drugs had MEAs extended repeatedly, without evaluation

- No information on reason for extension
- No information on RWE collected for reassessment
- No information on outcome of reassessment

- **Main focus on uncertainties concerning budget impact and efficacy data** (Graph 1)
- **Preference for mixed financial and outcome-based schemes** although lack of information on additional risk-sharing measures such as price discounts (Table 1)

RESULTS (CONTINUED)

Graph 1. The occurrence of uncertainties included in Belgian MEAs for orphan drugs

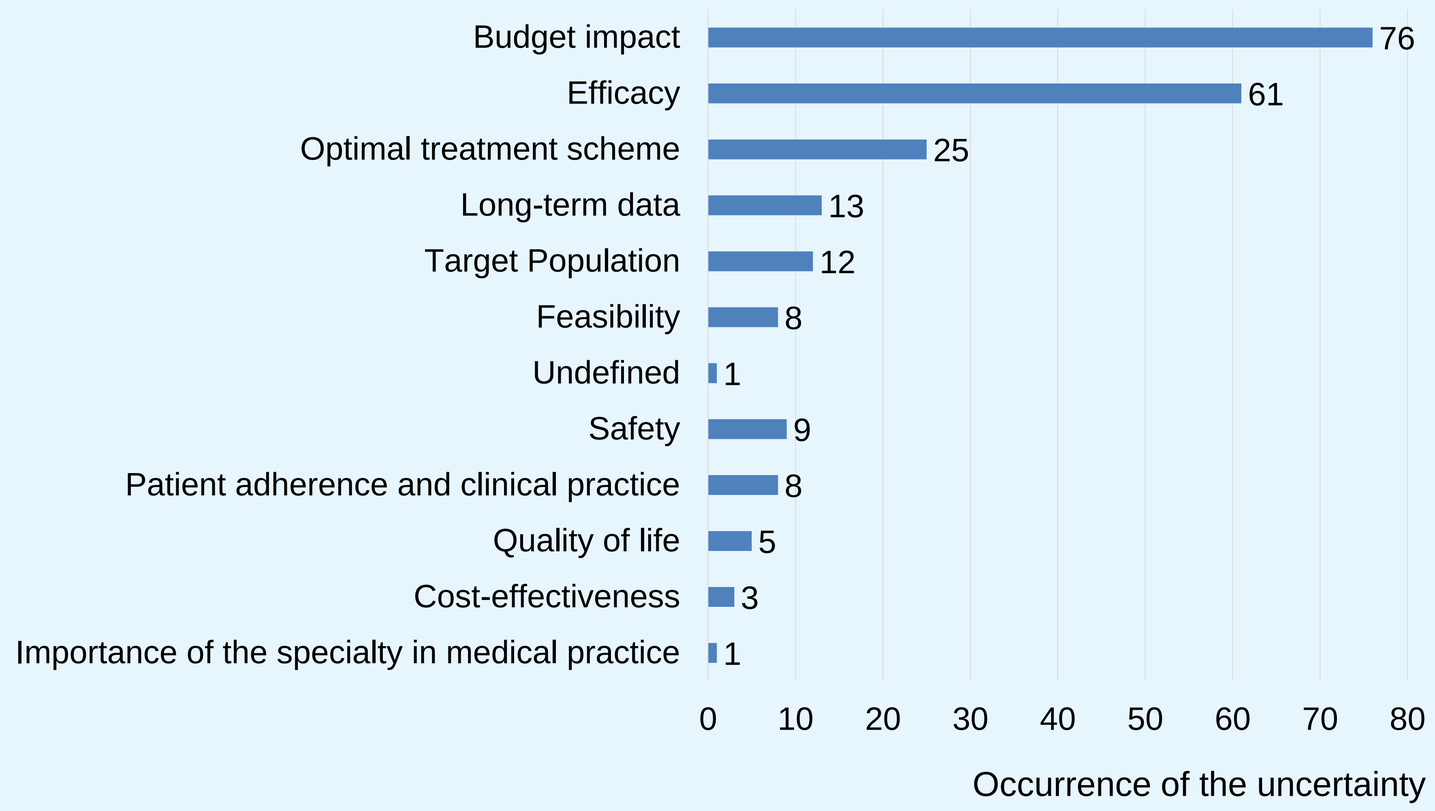

Table 1. Types of MEA schemes

Financial-based schemes	Outcome-based schemes
• MEAs for all orphan drugs contained measures for risk-sharing: market authorization holder (MAH) reimburses percentage of revenue	• MEAs for 19 of 31 orphan drugs contained measures for outcome-based schemes or Pay-for-performance
• Any additional risk-sharing measures such as price decreases or discounts, tailored to individual orphan drugs, were concealed from the public MEAs	• MEAs for 5 of 31 orphan drugs included requirements for the collection of RWE in registry

CONCLUSIONS

- Overall, the Belgian MEAs do not succeed at solving uncertainties
- None of the MEAs included information on RWE, so the use of RWE in Belgian MEAs remains a black box
- Lack of transparency on reassessments and measures taken impairs the justification of MEAs

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This poster was presented at ISPOR Europe 2022, Vienna, Austria

This research was funded by Fonds Wetenschappelijk Onderzoek (FWO)