

The Challenges of Outcomes-Based Reimbursement for Advanced Therapy Medicinal Products in Europe

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Introduction

- Advanced therapy medicinal products (ATMPs), such as cell and gene therapies, are often associated with single administration and curative potential in many chronic, life-limiting and life-threatening diseases.
- These ‘cures’ are typically accompanied by extremely high prices, while limited evidence to date means that their true long-term benefit is unknown.
- Outcomes-based reimbursement (OBR) is an attractive risk-sharing approach to the managed entry of ATMPs as it addresses both the inherent uncertainty in value determination and apprehensions regarding treatment cost.
- Despite the apparent utility of OBR, uptake remains low.
- The aim of this study was to review the literature discussing OBR for ATMPs in European markets to identify the barriers to, and opportunities for its use.

Methods

- A comprehensive scoping review of the literature published between Jan 2009 (date of first ATMP registration in Europe) – May 2022 was conducted.
- Literature sources included bibliographic databases indexing peer-reviewed journals of both biomedical and social sciences (Medline, Embase, Global Health, Health Management Information Consortium and the Cochrane Library) and grey literature.
- Grey literature searches centred on the websites of industry and patient organisations, and relevant authorities of the European Commission and individual countries responsible for the regulation of pharmaceutical products, management of pharmaceutical budgets or the appraisal of health technologies, in anticipation of published policy statements or information on ATMP funding decisions.
- Publications were included in the review if they discussed OBR agreements for ATMPs (or related factors) in European markets.
- Verbatim extracts from relevant discussions of OBR were captured using a standardised charting form and subsequently free-form coded by a single reviewer.
- Coded data were analysed for descriptive and overarching analytic themes representative of concepts describing the European OBR landscape using the methods described by Thomas and Harden (2008).
- A narrative synthesis was undertaken, which consisted of a descriptive summary of the coded information extracted from all publications and an exploration of the associations between prominent themes.

Results

- Bibliographic database searches returned 1,614 records and after removing duplicates, 536 publications remained for title and abstract screening.
- Of these publications, 205 were passed for full-text assessment, of which 74 were selected for inclusion in the review.
- Grey literature searches returned 57 records, of which ten were selected, giving a total of 84 articles included in the review. A combined PRISMA flow diagram is shown in Figure 1.
- Seventy-six per cent of articles included in the review were published within the last five years (2018–2022); of the records identified in the literature searches, no articles discussing OBR for cell, gene, or tissue-engineered therapies were published before 2013.
- Thirty-five of the 84 included publications had a Pan-European focus (42%), while 20 papers adopted a global perspective (24%). Publications that referred to a single European pharmaceutical market contributed to 14% (UK), 7% (Germany), 5% (Italy), 4% (France) and 1% (Spain, Switzerland, Sweden and Greece) of total knowledge.
- Content analysis of relevant text from each publication included in the review identified 15 descriptive themes within the literature, capturing a range of diverse factors, such as models of OBR application, structural features of health systems and pharmaceutical market dynamics (Figure 2).
- Descriptive themes were subsequently abstracted into six overarching analytical themes representing barriers to, and opportunities for, the increased use of outcomes-based agreements in the routine reimbursement of ATMPs: suitability and sustainability of innovative payment models, outcomes-based contract development, health system readiness, burden of administration, accelerated access to innovative medicines and conflicts of interest.
- A hypothetical model of relationships between analytical themes, based on prominence in the literature, is presented in Figure 3.

Conclusions

- Outcomes-based reimbursement may be a valuable tool in the development of a sustainable market for ATMPs.

- The proposed theoretical framework attempts to provide both an overview of the intricate, interrelating concepts governing the utility and adoption of OBR for ATMPs and a means of deconstructing the apparent complexity to allow specific barriers to be mitigated and opportunities realised in more focused settings.
- Payers and manufacturers should maintain awareness of market factors influencing OBR and anticipate the challenges and opportunities posed by each to facilitate early and effective dialogue between stakeholders in the cooperative implementation of OBR agreements.

References

- Thomas J and Harden A. Methods for the thematic synthesis of qualitative research in systematic reviews. BMC Medical Research Methodology. 2008. 8 (1):45

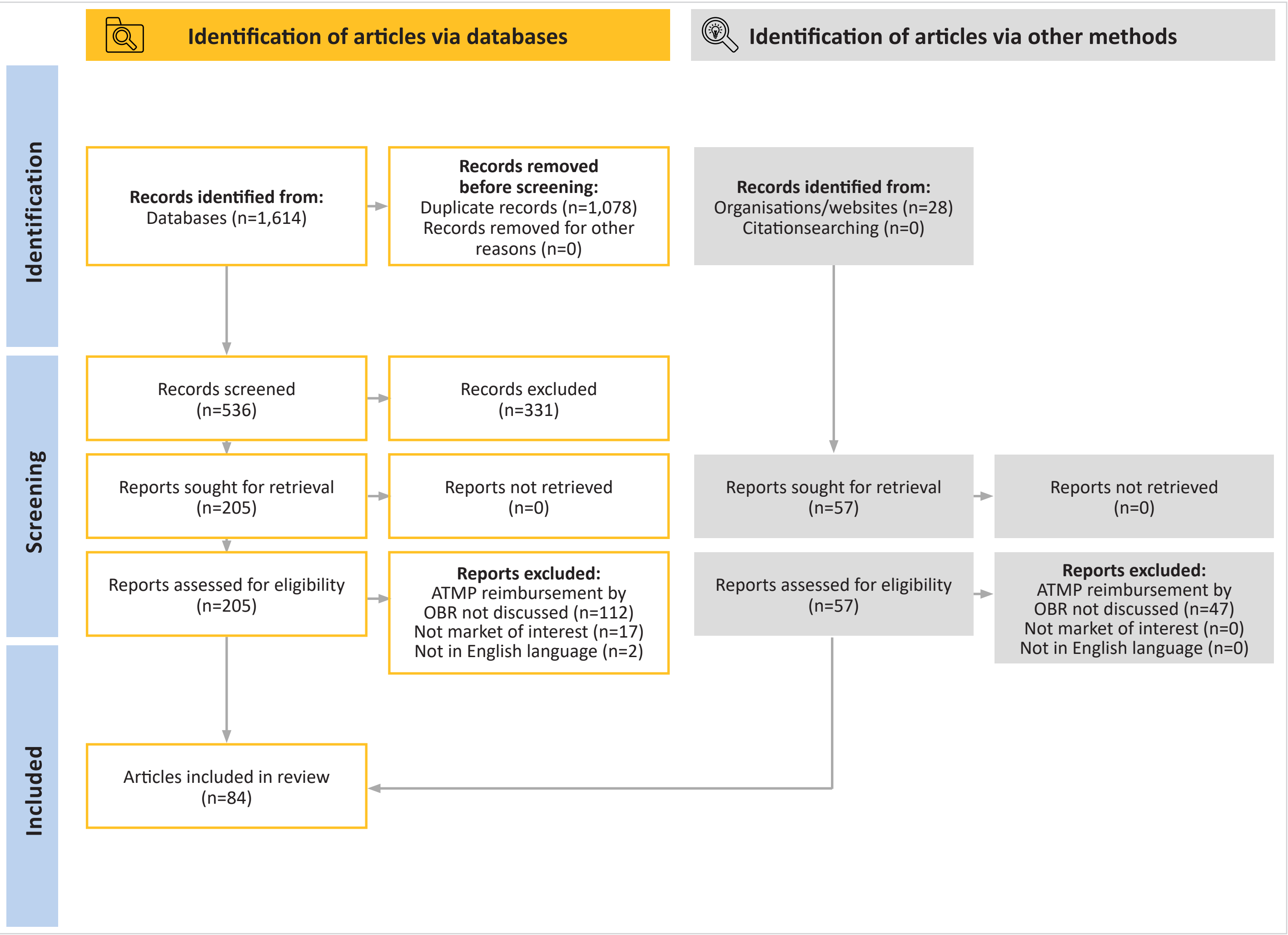


Figure 1. PRISMA diagram of the literature search and inclusion of publications.

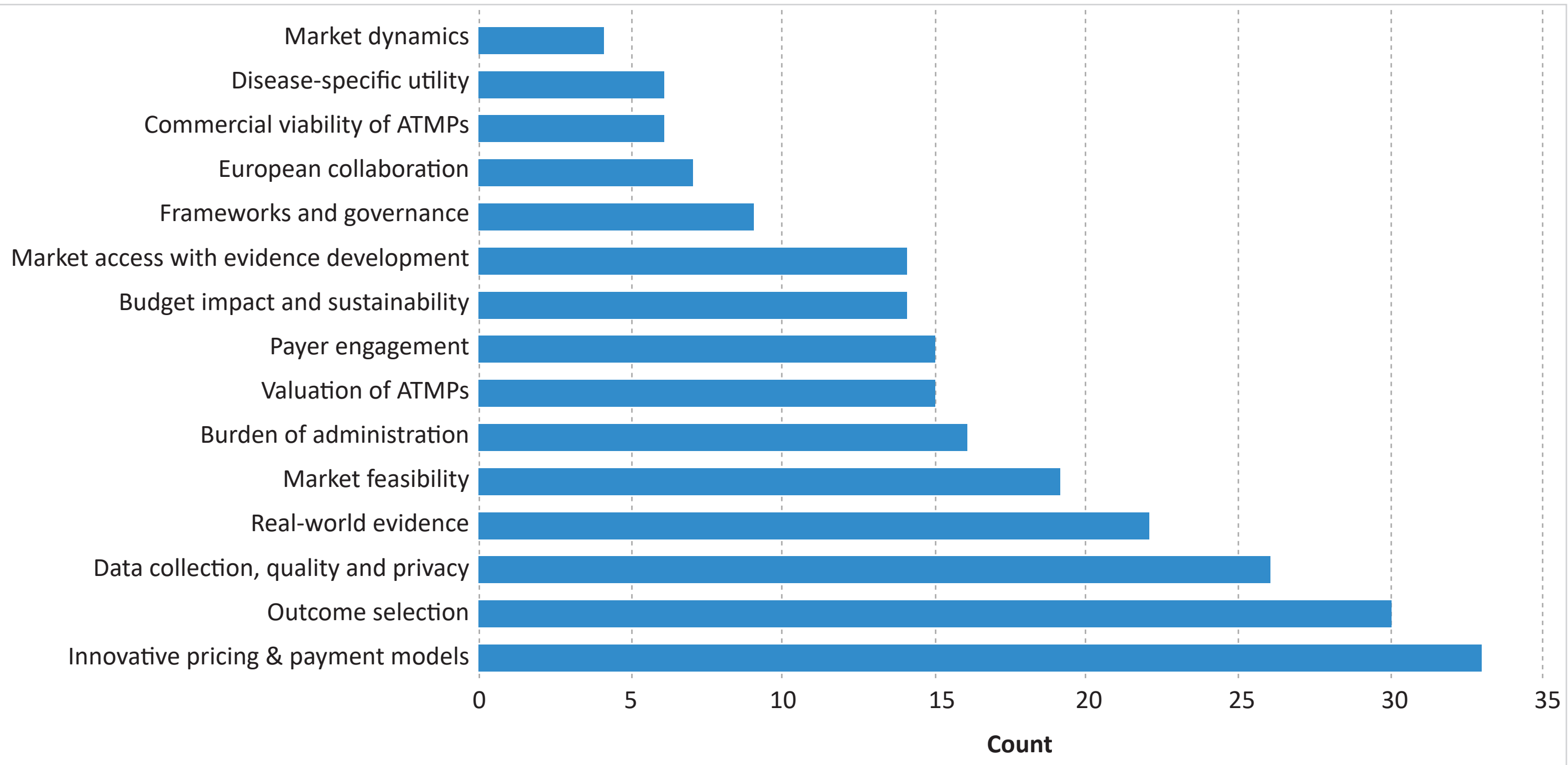


Figure 2. Factors associated with OBR of ATMPs by frequency in the literature.

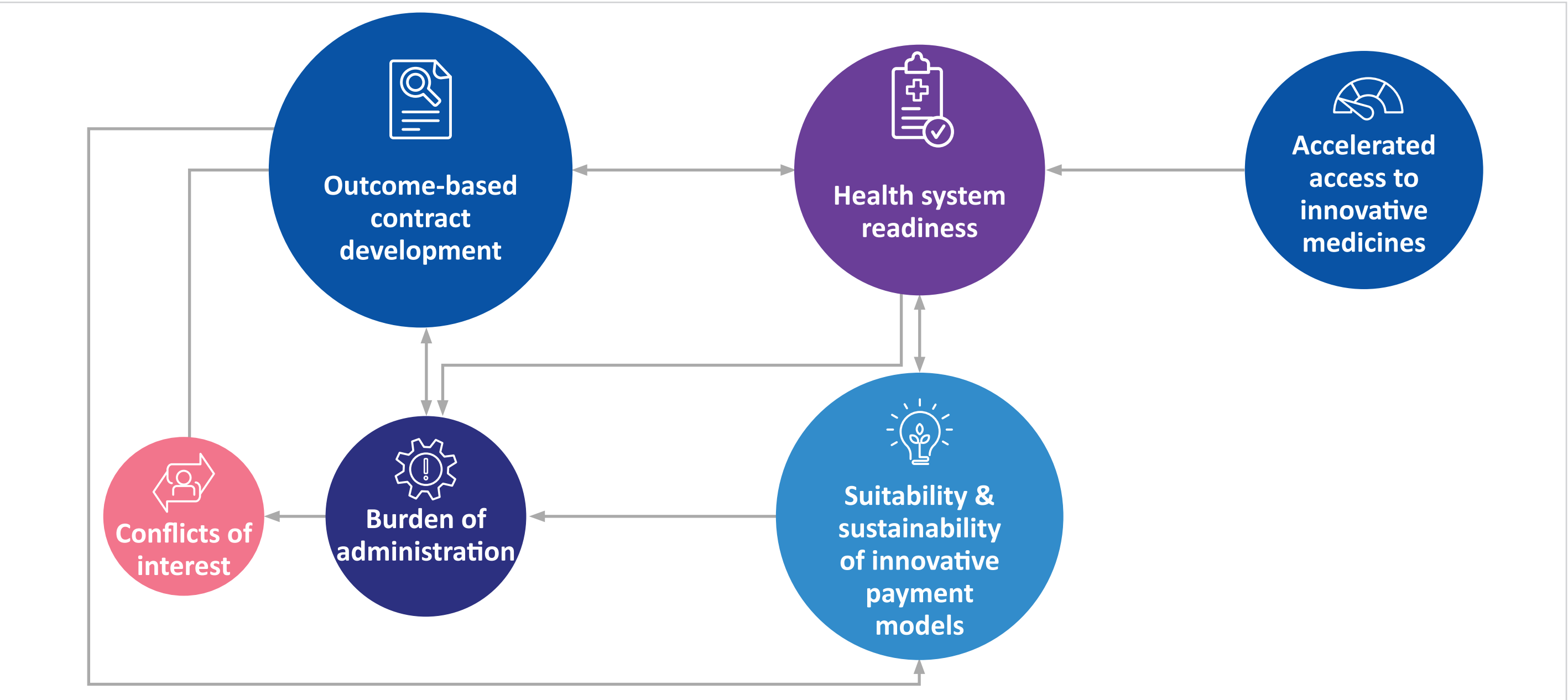


Figure 3. Hypothetical model of thematic relationships.

Single and double arrows represent unidirectional and bidirectional relationships, respectively. Circle size is indicative of relative significance, based on prominence in the literature.