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PROTOCOL

Mapping Payment and Pricing Schemes for Health Innovation: Protocol of a Scoping Literature Review

Vittoria Ardito[1] · Ludovico Cavallaro[1] · Michael Drummond[1,2] · Oriana Ciani[1]

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Abstract

Introduction Innovative pricing and payment/reimbursement schemes have been proposed as one part of the solution to the problem of patient access to new health technologies or to the uncertainty about their long-term effectiveness. As part of a Horizon Europe research project on health innovation next generation pricing and payment models (HI-PRIX), this protocol illustrates the conceptual and methodological steps related to a scoping review aiming at investigating nature and scope of pricing and payment/reimbursement schemes applied to, or proposed for, existing or new health technologies.

Methods A scoping review of literature will be performed according to the PRISMA guidelines for scoping reviews (PRISMA-ScR) guidelines. The search will be conducted in three scientific databases (i.e., PubMed, Web of Science, and Scopus), over a 2010–2023 timeframe. The search strategy is structured around two blocks of keywords, namely "pricing and payment/reimbursement schemes," and "innovativeness" (of the scheme type or scheme use). A simplified search will be replicated in the gray literature. Studies illustrating pricing and payment/reimbursement schemes with a sufficient level of details to explain their characteristics and functioning will be deemed eligible to be considered for data synthesis. Pricing and payment/reimbursement schemes will be classified according to several criteria, such as their purpose, nature, governance, data collection needs, and foreseen distribution of risk. The results will populate a publicly available online tool, the Pay-for-Innovation Observatory.

Discussion The findings of this review have the potential to offer a comprehensive toolkit with a variety of pricing and payment schemes to policymakers and manufacturers facing reimbursement and access decisions.

1 Introduction

1.1 Background

Medical innovation is advancing rapidly, but it is often characterized by clinical and economic uncertainty at the time of entry to the health care system. For medicinal products, clinical uncertainty is linked to the fact that often pivotal studies used for marketing approval do not follow the "gold standard" [i.e., blinded, two-arm, phase III randomized controlled trials (RCTs)] [1], or rely on surrogate endpoints as predictors of clinical effectiveness [2]. Regulatory agencies such as the European Medicines

Key Points

1. This protocol details the rationale and the methodology of a prospective scoping literature review aimed at mapping pricing and payment schemes for health innovation.

2. This work will inform on the different schemes available to promote access to potentially innovative, new or expensive health technologies in the area of medicinal products, medical devices, and drug–device combinations.

3. The pricing and payment schemes identified will populate an online, freely accessible tool named the Pay-for-Innovation Observatory, which will be made available to the scientific community.

✉ Vittoria Ardito
vittoria.ardito@sdabocconi.it

[1] Center for Research on Health and Social Care Management, SDA Bocconi School of Management, Via Sarfatti, 10, 20136 Milan, MI, Italy

[2] Centre for Health Economics, University of York, York, UK

Agency (EMA) and the Federal Drug Administration (FDA) are, therefore, granting marketing authorization on the basis of incomplete or limited evidence, sometimes with the commitment for the manufacturer to conduct postapproval clinical studies [2–5]. For medical devices (MDs), the quantity, type, and quality of evidence required for their approval has been traditionally considered to be weaker than for drugs [6, 7]. RCTs can often be not viable for MDs, characterized by unique features such as the incremental innovation or the learning curve associated with reiterated use. Similar considerations arise for drug–device combinations, including software-incorporating devices and digital medical devices, for which the incremental improvements of the device/software components can rapidly make past RCT results outdated. This scenario is often coupled with extremely high prices of certain health innovations, often resulting in large upfront payments, that occur before accrual of any clinical benefits and generate large budgetary impacts associated with reimbursement or coverage [8]. Considered together, this situation is posing significant challenges to authorities, payers, providers, and, ultimately, patients.

On top of this, access to health innovations can be further challenged by operational complexities raised by the distinctive features of certain technologies. These characteristics include new modes of administration (e.g., single-administration therapies), high treatment personalization, the need for a highly-skilled workforce, sophistication of the logistics of treatment delivery (e.g., transportation of lab-treated specimens), and difficulties in scaling up the manufacturing capacity due to the above. Consider, for example, Advanced Therapy Medicinal Products (ATMPs), often cited as paradigmatic examples of technologies that may combine all those challenges together [9, 10].

In this context, new pricing and payment/reimbursement models have been proposed as practical solutions to ensure timely patient access to promising innovations, while simultaneously addressing coverage problems. Innovative payment models (IPM) are agreements between manufacturers, governmental bodies, and payers defined to act as a bridge to access, reward research and development (R&D) efforts adequately, and balance the financial sustainability of healthcare systems [11]. New payment models have been termed differently and might be referred to as risk-sharing agreements (RSA) [12], managed entry agreements (MEAs) [13], or innovative contracting [14]. They might have a wide variety of formulations, with outcome-based and/or financial-based components or with payments split over time (e.g., instalments or annuities). For instance, a taxonomy developed by Carlson and colleagues categorized performance-based reimbursement schemes in terms of timing, execution, and health outcomes, distinguishing between outcome-based versus nonoutcome-based schemes [15]. In addition, Towse et al. distinguished between the agreements that specified how evidence would be translated into revisions of price, revenues, and/or use, and those that instead specified an evidence review point where renegotiation would take place [12]. Other frameworks have focused on coverage options more generally, distinguishing between schemes with objectives of evidence generation from those of price reduction [16], on different types of performance-based RSAs [17], or on the key reasons for using MEAs [18, 19]. More recently, Horrow and Kesselheim developed a taxonomy of possible payment arrangements for gene therapies, that include, among others, installments, subscriptions, expenditure caps, and others [8].

For clarity, we specify that this work will be focused on health technologies at large, including medicinal products, MDs, and drug–device combinations, and that these will be referred to interchangeably as "health technologies" or "health innovations." We also specify in this context the distinction between pricing and payment/reimbursement schemes. "Pricing schemes" refer to any approach or methodology to calculate, measure, or quantify a fair price for health technologies. An example is rate of return pricing, namely a scheme in which a prespecified rate of return is ensured to manufacturers, after covering the costs of developing and marketing the product [20]. On the other hand, "payment/reimbursement schemes" or arrangements refer to formulation of any aspect that has to be defined to govern the payment of health innovations, including, but not limited to, the types and number of stakeholders involved, the moment in which the payment occurs, the split of payments over time, or the linkage to an outcome component. Examples here include the subscription model, that delinks reimbursements from volumes of sales, offering manufacturers a fixed monetary amount [21], or the conditional treatment continuation agreement, where coverage is continued only for patients who achieve a prespecified response to treatment [13, 22, 23]. While the two approaches might capture the same value from different perspectives (i.e., manufacturers and payers), this is not always the case.

1.2 Objectives

Given the contemporary challenges experienced by healthcare systems globally in ensuring access to the latest available health technologies, the objective of this study is to perform an extensive mapping of the pricing and payment/reimbursement schemes that are currently used, or have been proposed, to allow for timely and widespread use of potentially innovative health technologies. Specifically, a scoping literature review will be conducted to respond to the following three research objectives:

1. To generate a comprehensive and updated catalogue of innovative pricing and payment/reimbursement schemes for health technologies;
2. To develop a conceptual framework that characterizes any pricing and payment/reimbursement schemes for health technologies, ultimately contributing to cluster them through a newly defined taxonomy;
3. To investigate which pricing and payment/reimbursement schemes are better suited to address a given coverage or reimbursement challenge, by accounting for the distinctive features of different technology classes, therapeutic areas, settings and healthcare systems, and ultimately clarifying which scheme best serves a given policy objective.

2 Methods

2.1 Protocol and Registration

This protocol was developed based on the PRISMA protocol guidelines and written in accordance with the PRISMA-P statement [24, 25]. The protocol has been registered in the International Prospective Register of Systematic Reviews (PROSPERO; registration number: CRD42023444824). The review will be conducted according to the updated methodological guidance and the PRISMA guidelines for scoping reviews (PRISMA-ScR) [26, 27]. Scoping reviews are a type of knowledge synthesis that follow a systematic approach to map relevant concepts, theories, sources, and knowledge gaps in a given area by extensively identifying, reviewing, and synthetizing the evidence available in literature [28].

2.2 Intervention

This scoping review will be focused both on pricing and payment/reimbursement schemes for health technologies as described above. Such schemes will be investigated across several dimensions relevant to their application, including technology classes, therapeutic areas, setting of care, healthcare systems, and geographies. These dimensions are described in more detail below.

2.3 Setting

Any pricing and payment/reimbursement schemes/strategy/arrangements that are used or that have been proposed for health technologies delivered either in-hospital or outpatient settings will be included. Within this perimeter, the focus is on technologies for which a pricing arrangement has to be established and negotiated with a manufacturer (i.e., external innovation). Conversely, innovations in services originated directly by health care providers (e.g., hospital-based innovation or innovation embedded in healthcare service delivery processes) will not be considered (i.e., internal innovation).

2.4 Timeframe

The timeframe of the current study will extend from 2010 onwards. Our search started in 2010 to build on the previously conducted study by Carlson et al. published in 2010, knowing that at the same time several countries started experimenting new schemes [15]. The literature search was performed in the first quarter of 2024 and will be updated in April 2024.

2.5 Eligibility Criteria

Studies illustrating pricing and payment/reimbursement schemes of health technologies with a level of detail that is sufficient to explain their functioning across different health technologies will be deemed eligible to be included in this review. Theoretical schemes (i.e., schemes that have only been proposed) and implemented schemes (i.e., schemes that have practical applications) will be equally considered in the analyses. Pricing and payment/reimbursement schemes will not be excluded based on their perceived innovativeness, as not only the scheme per se could be innovative but also the application or use in a given context. Furthermore, no exclusions will be made based on the country of implementation of the schemes, nor on the type of study design. For this reason, editorials, commentaries, and perspectives will be included when a given scheme is proposed and discussed. Search records will be extracted with no exclusions on the publication language, but the language expertise of the research team (e.g., English or Italian) will guide the study selection.

2.6 Information Sources

Literature searches will be conducted through different sources, and both scientific and gray literature will be considered.

Scientific publications will be searched in three databases, namely PubMed (Medline), Web of Science, and Scopus. In addition, the reference list of the studies included and of the reviews identified will be scanned to ensure that no relevant important work has been missed. In case relevant papers are not retrieved by our search, it will be replicated in top-tier journals in the area of pharmaceutical policy (i.e., Journal of Pharmaceutical Policy and Practice; Expert Review of Medical Devices; Expert

Review of Pharmacoeconomics and Outcome Research; Value in Health, European Journal of Health Economics; PharmacoEconomics; PharmacoEconomics—Open; Health Economics; Applied Health Economics; Health Policy; Health Affairs; Applied Health Services Research and Policy; Cost-effectiveness and Resource Allocation).

As for the gray literature, reports, white papers, and websites of a range of relevant institutions will be searched. Key institutions include, but are not limited to, international organizations, industry-oriented organizations, HTA agencies, patient associations, and consulting and research companies, such as European Commission (EC), Organization for Economic Cooperation and Development (OECD), European Federation of Pharmaceutical Industries and Associations (EFPIA), International HTA Database, Pharmaceutical Pricing and Reimbursement Information (PPRI), European Patient Forum (EPF), European Patients Academy for Therapeutic Innovation (EUPATI), and others. As a subsequent step, the list of schemes identified will be circulated to the relevant individuals in HTA bodies and other relevant institutions mentioned above, in case they are aware of any that have not been identified.

2.7 Search Strategy

The structure of the search strategy is developed around two main concepts: (1) "pricing and payment/reimbursement schemes" and (2) "innovativeness" (of the scheme type or scheme use). Particularly, it is built using combinations of the following terms: performance-based, value-based, evidence-based, risk-sharing, reimbursement, rebate, pricing, contract, scheme, guarantee, and health system. To restrict the number of retrieved records, database-specific addendums are used to filter the two main search blocks, namely Mesh Terms in PubMed (Medline), Web of Science categories in Web of Science, and index terms in Scopus. The complete search for each database is presented in Table 1.

2.8 Study Records

2.8.1 Study Selection

The records retrieved through the database search will be imported in RefWorks, a tool for reference management that is used to detect and remove duplicated studies. The final list of records will be exported into a structured Microsoft Excel spreadsheet, where they will be screened based on title and abstract, and assessed against eligibility criteria. Two members of the research team (V.A. and L.C.) will assess the first 200 records based on title and abstract, and the inter-rater agreement will be measured using kappa statistics [29].

Table 1 Search query for PubMed, Web of Science, and Scopus

	PubMed (Medline)	Web of Science	Scopus
Search block 1	[Title/abstract] reimburs* OR pric* OR payment OR coverage OR rebate OR discount OR subscription OR fee OR installment OR tariff OR financing model		
Search block 2	[Title/abstract] performance-based OR performance-oriented OR outcome-based OR outcome-oriented OR result-driven OR value-based OR evidence-based OR financial-based OR managed OR risk-sharing OR adaptive OR innov* OR new OR promising OR dynamic		
Database-specific addendums	Mesh terms: Economics, pharmaceutical OR economics, medical OR insurance, health, reimbursement OR costs and cost analysis OR drug costs	Web of Science Categories: (Health Care Sciences & Services OR Health Policy & Services OR Pharmacology & Pharmacy OR Chemistry, Medicinal OR Engineering, Biomedical OR Medicine, Research & Experimental)) AND (Economics OR Management OR Political Science OR Social Sciences, Biomedical)	Index terms: economics, pharmaceuticals; economics, medical; insurance, health, reimbursement; cost and cost analysis; drug cost

The remaining papers will be first screened based on title and abstract and then read full/text by two researchers. Disagreement over final inclusions will be solved by an arbitrator (O.C.). The entire research team will read all the studies eventually included in the analysis.

2.8.2 Data Collection Process

Data collection will be performed by two independent researchers (V.A. and L.C.). Data will be extracted using an ad hoc Microsoft spreadsheet, developed by the research team after preliminarily reading a pool of seminal papers. To ensure consistency across reviewers, the extraction sheet will be tested by each reviewer and possibly recalibrated before starting the data collection process. Information on the pricing and payment/reimbursement schemes will be collected, as specified in the following section.

2.8.3 Data Extraction

Data items will be collected at the individual scheme level, although different studies may contribute to the definition of a single scheme. Data items to be extracted may include general information on the scheme, and information on one or more examples of implementation, if available, as indicated in the following Table 2.

2.8.4 Data Synthesis

The study findings will be synthetized using narrative synthesis. Descriptive statistics on the pricing and payment/reimbursement schemes identified through this review will be provided, according to the most relevant dimensions of the data collection. Given the exploratory nature of this scoping review and the variety of the types of studies (expected to be predominantly studies with qualitative designs), a quantitative synthesis of the results will not be performed. Furthermore, given the foreseen high variety of the studies, a risk of bias assessment will not be performed. In parallel, the catalogue of pricing and payment/reimbursement schemes mapped through the review will be made accessible online to the scientific community in the form of a freely available repository called the Pay-for-Innovation Observatory, that different stakeholders could use for a variety of purposes. This broad availability of the findings of the review will also facilitate constructive comments and feedback.

2.9 Machine Learning-Powered Updates of the Scoping Review

Considering the rapidly evolving landscape of health innovations and the ensuing pricing and payment challenges, our work will be periodically updated with ASReview (https://asreview.nl/), an open-source machine learning (ML) software that allows to streamline the screening process for titles and abstracts within systematic reviews. In addition to the primary search, the ML-based software will be employed to perform periodic updates of the scoping review. ASReview utilizes an active researcher-in-the-loop ML algorithm, employing text mining to rank articles in terms of their likelihood for inclusion. This approach involves prior human input from the research team to guide the ML screening process and decision. ASReview offers various classifier models to determine the relevance of included articles. In a simulation study using six comprehensive systematic review datasets covering diverse topics, it was observed that the naive Bayes (NB) and term frequency-inverse document frequency (TF-IDF) models outperformed other settings [30]. The NB classifier estimates an article's relevance probability based on TF-IDF measurements, which gauge the uniqueness of specific words within an article relative to their frequency across all articles [31]. Consequently, the combination of NB and TF-IDF has been selected for use in our work.

The software will be trained using at least one relevant and one irrelevant article to establish a foundational knowledge base, with the expectation that performance will be enhanced as prior knowledge increases.

ASReview will conduct an initial ranking of all unlabeled articles, sorting them based on descending probabilities of relevance. The top-ranked article will undergo assessment of its title and abstract against the predetermined eligibility criteria, thereby determining its relevance. Following this assessment, the ML tool will assimilate the acquired knowledge and recalibrate the article rankings, with the subsequent highest-ranked article being presented for evaluation against the eligibility criteria. This iterative interplay between the ML tool's ranking and the reviewers' decision making continues until reaching a data-driven stopping criterion previously defined by the research team, i.e., the sampling criterion (which entails screening a set proportion of the highest-ranked articles) and the heuristic criterion (which prompts screening cessation upon encountering *n* consecutive predefined irrelevant articles).

3 Discussion

This scoping review of literature aims at investigating innovative pricing and payment/reimbursement schemes for health technologies, as well as at exploring innovative ways of using established schemes (e.g., price–volume agreements). This work will be conducted as part of the larger Horizon Europe research project Health Innovation Next Generation Payment and Pricing Model (HI-PRIX; grant agreement number 101095593), which aims at fostering access to health innovations by promoting the adoption of

Table 2 Comprehensive list of data items for data extraction

Data items	Data item description
1. General information on the scheme:	
Scheme name	Identification of each scheme with its given name, when available, or with an identifying label or expression. Both names translated in English and in the original language will be provided.
Scheme description	High-level description of the defining elements of the scheme, its model of functioning, the stakeholders involved and their role, and other distinguishing features
Scheme objective	Description of the main rationale(s) associated with a given scheme that identifies the intended policy objectives that the stakeholders involved are willing to achieve by using the scheme
Type of scheme	Classification of each scheme as "pricing scheme" or "payment/reimbursement scheme," subject to whether it refers to any approach to define the price of health technologies, or to any approach to defined how to pay for new or expensive health products
Theoretical versus applied scheme	Classification of each scheme as "theoretical" (i.e., indicating that it has only been proposed or theorized in the literature) or as "applied" (i.e., indicating that it has been implemented and that it is used in real-world contexts)
Perspective	Classification of a given scheme as "patient level" or "population level," subject to whether the mechanisms that trigger financial or outcome-based aspects of the scheme are defined at the patient or population level, respectively
Distribution of risk	For schemes that aim at managing at-launch uncertainties around financial or outcome-based dimensions of the scheme, indication of how such risk is shared amongst the parties involved (e.g., manufacturers, authorities, and payers)
2. In case the scheme has been implemented in real life:	
Case of application	Example of application of a given scheme in a real-world context, when available. This could be identified by either using the brand name and/or active principle for drugs; the brand name, for medical devices; or other varying labels that identify a given case of application
Case of application description	Detailed description of the case of application, including its key elements, its functioning, the stakeholders involved, and other distinguishing features
Country	Indication of the country or countries where the case of application has been implemented
Date	Indication of the date when the case of application under analysis was first implemented
Status of the scheme	Indication of the status of the scheme at the time of data collection, namely whether it is "closed," "ongoing," "yet to start," or "other"
Length/time horizon	Indication of the timeframe of validity of the case of application, namely its duration or the extension over time
Product category	Classification of the case of application based on the type of health technology under analysis, clustering the schemes as being applied to "drugs," "medical devices," "digital technologies," or combination of these technologies
Drug type (if product category is drugs)	When the product category being considered is "drugs," classification of the case of application based on macrocategories of drug types, clustering the schemes as being applied to "patented drugs," "generic drugs," "vaccines," "antibiotics," "ATMPs/gene therapies," or "others"
Device type (if product category is devices)	When the product category being considered is "devices," classification of the case of application based on macrocategories of device types, clustering the schemes as being applied to "diagnostics," "in vitro diagnostics" or "others"
Therapeutic area	Indication of the therapeutic area of the health innovation object of the case of application
Mode of administration	Classification of the case of application based on the mode of administration of a given therapy, clustering the schemes as "single administration" (e.g., for gene therapies), "cycles of treatment" (e.g., for oncologic drugs), or "life long" (e.g., for chronic diseases)
Setting	Identification of the setting of care in which the case of application is delivered, clustering the schemes as "inpatient," "outpatient," or "other"
Healthcare system	Indication of the type of healthcare system where the case of application is implemented, typically distinguishing between "tax based" or "insurance based" systems

Table 2 (continued)

Data items	Data item description
Data collection needs	For the performance-based schemes, indication of the needs for data collection, the type of study used in the evaluation, the type of data to be collected, the health outcome measure (for outcome-based schemes), and the outcome of the scheme (e.g., coverage and reimbursement consequences)
Governance of the scheme	Indication of the responsibilities for governance, data collection and analysis of the scheme

new pricing and payment models, in an effort to balance sustainability of health innovation with sustainability of healthcare systems. The findings of this review will be made freely accessible to the scientific community that includes governmental bodies, payers, HTA agencies, and policy makers, through an online tool, which will be termed the Pay-for-Innovation Observatory. Other databases already exist, such as the Performance Based Risk Sharing Database, proprietary of the University of Washington [32], or the repository on medical devices produced as part of the EU's Horizon 2020 research project Pushing the Boundaries of Cost and Outcome Analysis of Medical Technologies (COMED). Our Pay-for-Innovation Observatory will build on these prior examples, expanding on the dimensions investigated (e.g., classes of health technologies covered) and making the database openly accessible.

Previously published taxonomies have classified pricing or payment/reimbursement schemes with a siloed approach, typically focusing separately on clusters of schemes, such as performance-based risk sharing agreements RSAs only [17], MEAs only [18, 19], or coverage with evidence development (CED) schemes only. Furthermore, prior taxonomies have been predominantly developed using the lens of the public authorities or payers [15, 16, 23], as these mostly categorize the available coverage options as opposed to the strategies available to manufacturers to price health technologies. Lastly, these previous frameworks were published mostly in the early 2010s (i.e., the majority before 2014) and might fail at accounting for some of the innovative contracting schemes that have been designed to address the distinctive features of new health technologies, such gene therapies, that have now become available.

All in all, this work will inform on the different schemes available to promote access to potentially innovative, new or expensive health technologies in the area of medicinal products, medical devices, and drug–device combinations.

Data Availability Data sharing is not applicable to this article as no datasets were generated or analyzed during the current study.

Code Availability Not applicable.

Declarations

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Conflict of Interest V.A., L.C., M.D., and O.C. declare that they have no conflict of interest.

Authors' Contribution V.A. and O.C. contributed to the study conception. V.A. drafted the first version of the manuscript. V.A. and L.C. designed the preliminary search strategy and performed the initial screening of the records. All authors (V.A., L.C., M.D., and O.C.) contributed to developing the data extraction form and revised and approved the final version of this manuscript.

Ethics Approval Not applicable.

Consent to Participate Not applicable.

Consent for Publication (from patients/participants) Not applicable.

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