RESEARCH ROUNDUP

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In keeping with the theme of this issue, we've tried to identify recent research publications that highlight innovative pricing models for pharmaceuticals and/or medical devices. There is a large body of editorial, commentary, and promotional publications, but sparse research, and so in the end it was not an easy task to select the papers for this round. However, there have been some recently published empirical research and conceptualized frameworks, and we have identified 5 research papers that are worth reading. We hope the research highlighted will contribute to a discussion and debate about innovation, pharmaceuticals, medical devices, and pricing.

Innovative payment models for high-cost innovative medicines: report of the expert panel on effective ways of investing in health

European Commission. European Union, 2018. Reuse is authorized provided the source is acknowledged. https://ec.europa.eu/health/expert_panel/sites/expertpanel/files/docsdir/opinion_innovative_medicines_en.pdf Accessed April 26, 2020.

Summary

The premise of this report from the expert panel recognizes that the current path of growth of pharmaceutical expenditures due to new high-cost innovative medicines cannot be continued indefinitely. The report also identifies the need to search for new ways to ensure that innovation "that matters" is produced, that patients have access to innovation, and that health systems are financially sustainable. It is in this context that the report leads to the discussion of innovative payment models for new medicines that could improve the way the objectives are met.

A single payment model is unlikely to be optimal for all situations, and the report outlines some broad principles that should be observed when defining specific payment models for innovative medicines and deciding on rewarding research and development in pharmaceutical products.

Relevance

A variety of different pricing models are proposed and no single payment model emerges as dominant, but this does not preclude that clusters of models will develop over time. It is probable that different countries and systems will learn from each other's experience, and the policy toolbox will make use of several payment models, according to the most relevant problem in each case. The authors provide a detailed report that's worth reading more than once.

Outcomes-based reimbursement for gene therapies in practice: the experience of recently launched CAR-T cell therapies in major European countries

Jørgensena J, Hannab E, Kefalasa P. *J Mark Access Health Policy*. 2020;8(1715536):doi.org/10.1080/20016689.2020.1715536

Summary

This research provides an overview of the reimbursement schemes used for 2 novel and innovative cancer treatments, the chimeric antigen receptor T (CAR-T) cell therapies, Kymriah® (tisagenlecleucel) and Yescarta® (axicabtagene ciloleucel) in France, Germany, Italy, Spain, and the United Kingdom (EU5) as per the final quarter of 2019. The study also identifies the challenges and derives learnings about how other advanced therapy medicinal products may be adopted in the future. Both products have successfully obtained reimbursement in their labelled indications across the EU5, at relatively uniform list prices, and the paper describes in detail each country's outcomes-based reimbursement scenarios. But it should be noted that the prices detailed reflect the list prices and do not (necessarily) reflect the actual amount paid once rebates, discounts, or performance-based payment mechanisms have been accounted for.

Relevance

This paper highlights how innovative, high-cost therapies with data uncertainty at launch, and with the potential to deliver significant patient and healthcare system benefits, can secure reimbursement and adoption via novel examples of outcomes-based reimbursement with the staged payments tied to patient outcomes such as those used for CAR-T cell therapies. The paper is well worth a read to explore the various novel approaches to reimbursement being applied.

Defining the concept of fair pricing for medicines

Moon S, Mariat S, Kamae I, Bak Pedersen H. *BMJ*. 2020;368(l4726):dx.doi.org/10.1136/bmj.l4726

Summary

In this research, Moon and colleagues consider what makes a fair price for both buyers and sellers of medicines and describe a conceptual framework for assessing whether a medicine's price is fair to each. The authors identified 4 categories to be considered when assessing fairness for sellers, and 3 categories of demand-side factors for the buyers, and combined the factors into a framework in which a fair-pricing zone lies between a price floor and ceiling. The price floor is the lowest sustainable price at which suppliers can sell a medicine. The price ceiling is the maximum the buyer can afford. Prices above the ceiling are defined as excessive and would justify regulation. A fair price for a medicine is affordable to the buyer while covering the seller's costs and providing a reasonable profit margin. Within a fair-pricing zone, a specific price may be higher or lower, possibly reflecting value or distribution of consumer and producer surplus.

Relevance
This framework does not fix a fair price for a medicine through a cost plus formula but instead, it provides a way of systematically assessing whether any given price is fair by taking costs into account. The framework argues for a concept of pricing that explicitly takes into account the needs of both sellers and buyers, and the broader public interest objectives of securing innovation, sustainable supply, and affordability. Applying the framework to decision making, however, would require access to data on research and development, manufacturing, and distribution costs, which may limit its applicability.

The price of innovation—the role of drug pricing in financing pharmaceutical innovation: a conceptual framework

Morenoa SG, Epstein D.
J Mark Access Health Policy. 2019;7(1583536):doi.org/10.1080/20016689.2019.1583536

Summary
The aim of the research was to describe how the pharmaceutical industry finances innovation, and how deviations from the principles of value-based pricing (either by industry or by payers) can distort access to capital markets and lead to undesirable outcomes for patients, healthcare systems, and ultimately society at large.

The authors propose a conceptual framework describing the mechanism that links investors in capital markets to pharmaceutical innovation. The framework describes, from a financial perspective, the role played by key features along the life cycle of pharmaceutical innovation and the role that drug prices play in influencing the ability of pharmaceutical firms to raise money in capital markets and hence, finance pharmaceutical innovation. The framework breaks up the mechanism leading to innovation into a loop of 4 causal associations.

Relevance
The framework may be able to help policymakers appreciate the life cycle of innovation from a financial perspective and inform future policy proposals in the area of drug pricing. The framework may also help policymakers anticipate the impact of their proposals and ultimately guide policies towards setting optimal drug prices as a means to maximize social welfare.

In the end, this research contributes to the much-needed debate about the role of drug prices in incentivizing innovation.

Reimbursement pricing for new medical devices in Japan: is the evaluation of innovation appropriate?

Tamura M, Nakano S, Sugahara T.
Int J Health Plann Mgmt. 2019;34(583–593):doi.org/10.1002/hpm.2719

Summary
This research assesses whether the evaluation of innovation in medical devices in Japan is appropriate, and compares the reimbursement process and issues between several product categories to illustrate this point. Detailed discussion on the overview of Japan's medical device reimbursement policy and the price-setting rules and methodology are outlined. The paper specifically looks at 2 major types of reimbursement rules for medical devices: the rule determining the prices for individual medical devices (STM), and the rule incorporating the price as part of the technical fee for diagnostic devices (nonSTM). The research indicates that innovation evaluation gradually declined, and the authors explain the main reasons for this.

Relevance
The research provides a very detailed and empirical insight into the issues and the related policy reform for medical device reimbursement in Japan. In order to understand medical device pricing and reimbursement and the limitations in Japan, this paper is a must-read.