VALUE IN HEALTH 16 (2013) 703–719

Available online at www.sciencedirect.com

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journal homepage: www.elsevier.com/locate/jval

ISPOR TASK FORCE REPORTS

Performance-Based Risk-Sharing Arrangements—Good Practices for Design, Implementation, and Evaluation: Report of the ISPOR Good Practices for Performance-Based Risk-Sharing Arrangements Task Force

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ABSTRACT

There is a significant and growing interest among both payers and producers of medical products for agreements that involve a "pay-for-performance" or "risk-sharing" element. These payment schemes—called "performance-based risk-sharing arrangements" (PBRSAs)—involve a plan by which the performance of the product is tracked in a defined patient population over a specified period of time and the amount or level of reimbursement is based on the health and cost outcomes achieved. There has always been considerable uncertainty at product launch about the ultimate real-world clinical and economic performance of new products, but this appears to have increased in recent years. PBRSAs represent one mechanism for reducing this uncertainty through greater investment in evidence collection while a technology is used within a health care system. The objective of this Task Force report was to set out the standards that should be applied to "good practices"—both research and operational—in the use of a PBRSA, encompassing questions around the desirability, design, implementation, and evaluation of such an arrangement. This report provides practical recommendations for the development and application of state-of-the-art methods to be used when considering, using, or reviewing PBRSAs. Key findings and recommendations include the following. Additional evidence collection is costly, and there are numerous barriers to establishing viable and cost-effective PBRSAs: negotiation, monitoring, and evaluation costs can be substantial. For good research practice in PBRSAs, it is critical to match the appropriate study and research design to the uncertainties being addressed. Good governance processes are also essential. The information generated as part of PBRSAs has public good aspects, bringing ethical and professional obligations, which need to be considered from a policy perspective. The societal desirability of a particular PBRSA is fundamentally an issue as to whether the cost of additional data collection is justified by the benefits of improved resource allocation decisions afforded by the additional evidence generated and the accompanying reduction in uncertainty. The *ex post* evaluation of a PBRSA should, however, be a multidimensional exercise that assesses many aspects, including not only the impact on long-term cost-effectiveness and whether appropriate evidence was generated but also process indicators, such as whether and how the evidence was used in coverage or reimbursement decisions, whether budget and time were appropriate, and whether the governance arrangements worked well. There is an important gap in the literature of structured ex post evaluation of PBRSAs. As an innovation in and of themselves, PBRSAs should also be evaluated from a long-run societal perspective in terms of their impact on dynamic efficiency (eliciting the optimal amount of innovation).

Keywords: access with evidence development, conditional licensing, coverage with evidence development, managed entry schemes, outcomes-based, patient access schemes, pay for performance, performance-based risk-sharing arrangements, risk-sharing.

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1098-3015/$36.00 – see front matter

http://dx.doi.org/10.1016/j.jval.2013.04.011

Background to the Task Force

Since 2007, there has been an acceleration in interest in a variety of arrangements between medical product manufacturers and payers that tie postlaunch data collection to payments. The ISPOR Performance-Based Risk-Sharing Arrangements Good Practices Task Force was approved by the ISPOR Board of Directors in March 2011 to set out the standards that should be applied to these arrangements, encompassing the design, implementation, and evaluation of such agreements. The report builds on previous work undertaken at Banff, in the UK Pharmaceutical Price Regulation Scheme, and by others as well as relevant work undertaken by other ISPOR Good Research Practice Task Forces, notably those tackling issues around the design, collection, and use of observational data to improve the quality of decision making.

Professors Lou Garrison and Adrian Towse, task force co-chairs, chose task force members on the basis of their knowledge and experience in decision modeling, study design, market access, coverage with evidence development, and performance-based pricing arrangements. Members represented a diverse range of practice and perspectives, including government (Agenzia Italiana del Farmaco [AIFA]), academia, health economic research and policy organizations, as well as the pharmaceutical industry. The task force was international with members from France, Italy, The Netherlands, Switzerland, the United Kingdom, and the United States.

The Task Force met approximately once a month by teleconference to develop and revise the outline and draft, as well as to discuss issues that arose in the process. A face-to-face meeting was held in November 2011 to develop recommendations and to reach consensus on content issues. In addition, the task force chairs had a series of one-on-one teleconferences to revise sections of the manuscript. All task force members reviewed and provided frequent feedback via oral or written comments on the manuscript drafts.

Preliminary findings were presented in a forum at the 2011 ISPOR 14th Annual European Congress in Madrid, Spain. Updated findings were presented at the Third Plenary Session of the ISPOR 17th Annual International Meeting in June 2012 in Washington, DC. In addition to the oral comments received during the two presentations, a draft of this article was distributed to the 100+ person ISPOR Performance-Based Risk-Sharing Arrangements Task Force Review Group in January 2012. During the Review Group round of comments and the final manuscript review sent to the entire ISPOR membership, several hundred written comments were received from 104 ISPOR members and organizations.

All comments, most of which were substantive and constructive, were considered. The comments were reviewed and discussed by the task force in a series of teleconferences, and addressed as appropriate in a revised manuscript. Once consensus was reached by all authors, the final report was submitted to *Value in Health* in April 2013.

All written comments, as well as the task force's responses, are published at the ISPOR Web site on the task force's Web page: http://www.ispor.org/Taskforces/performance-based-risk-sharing-arrangements.asp The task force report and Web page may also be accessed via the ISPOR homepage (www.ispor.org) via the purple Research Tools menu, Good Practices for Out comes Research. A list of reviewers is also available via the task force's Web page.

Introduction

There is a significant and growing interest among payers and producers of medical products for agreements that involve a "pay-for-performance" or "risk-sharing" element. These payment schemes—called "performance-based risk-sharing arrangements" (PBRSAs)—involve a plan by which the performance of the product is tracked in a defined patient population over a specified period of time and the level or continuation of reimbursement is based on the health and economic outcomes achieved. One database study identified 116 cases of these types of arrangements for medicines and other medical products since 1997 [1], with slowly growing numbers in the most recent years. (See [2] and [3] for comprehensive lists of PBRSA examples.) This broad trend across many developed countries represents, in part, a response to the growing cost of new drugs and other innovative medical products and the desire of payers to obtain greater certainty and greater value for the money spent.

There has always been considerable uncertainty at product launch about the ultimate real-world clinical and economic performance of new medical products. The uncertainty and concomitant financial risk to the payer for a new treatment that does not work as anticipated in the real world has increased along with the rising price of the new treatments, whether a biologic, device, or other medical technology. If payers are reluctant to adopt, manufacturers face the risk of reduced revenue for a product they regard as delivering value. PBRSAs represent one mechanism for reducing uncertainty through greater investment in evidence collection while a technology is in use within a health care system.

Information about what works in medical care is, in economic terminology, a public good—one person's use of the information generally does not keep others from using it—regardless of whether it is generated by public or private entities. Public authorities who negotiate and fund evidence-generating arrangements need to follow good research practices (GRPs) to improve the quality of the information derived and to make the results of that research public where possible. Private insurers, who may have less legal obligation for transparency, can still benefit from GRPs as they seek valid scientific answers to the outcomes questions embedded in the arrangements they negotiate. Encouraging them to put their findings in the public domain can generate greater public benefit as well, as long as it does not inappropriately deter them from agreeing to PBRSAs.

The objective of this Task Force report was to set out the standards that should be applied to "good practices"—both research and operational—in the use of a PBRSA, encompassing questions around the desirability, design, implementation, and evaluation of such an arrangement. This report provides practical recommendations for the development and application of state-of-the-art methods to be used when considering, using, or reviewing PBRSAs.

Defining PBRSAs

PBRSAs fall under a variety of names and categories: outcomes-based schemes, risk-sharing agreements, coverage with evidence development (CED), access with evidence development, patient access schemes (PASs), conditional licensing, and managed entry schemes [2,4–10]. For the purposes of this discussion, we group all these under the broad term "performance-based risk-sharing arrangements" (PBRSAs).