

**TASK FORCE**



**Developing Good Research Practices for Performance-Based Risk-Sharing Arrangements**




**ISPOR Performance-Based Risk-Sharing Task Force**



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**Purpose of the Session**

- Provide an update on Task Force objectives and progress.
- Identify and describe key issues and challenges.
- Seek audience input and comments.



## Outline of the Session

- Task Force Objectives
- What is a PBRSA?
- Distinguishing among PBRsAs
- The Four Key research questions
- Desirability of PBRsAs
- Approaching Research Design
- Timing
- Implementation
- Evaluation
- Governance
- Public vs. Private Payers



## Task Force Objectives

- To produce a report that sets out the standards that should be applied to a “good practice” PBRSA, encompassing the design, implementation, and evaluation of such an arrangement.
- To provide practical recommendations for the development and application of state-of-the-art methods to be used in the design and evaluation of PBRsAs.



## What is a PBRSA?

- PBRSA = Performance-Based Risk Sharing Arrangement
- Also known as:
  - outcomes-based schemes
  - risk-sharing agreements
  - coverage with evidence development
  - access with evidence development
  - patient access schemes
  - conditional licensing
  - managed entry schemes
  - pay-for-performance programs
  - innovative pricing
  - and others?



## PBRSA—Working Definition (1)

1. There is a program of data collection—either by agreement between the manufacturer and payer or initiated by the payer— to reduce uncertainty about the expected effectiveness (including possible negative effects) and cost-effectiveness of the drug (or device or diagnostic) or other relevant outcome or cost impact on the health care system.
2. Data collection is typically initiated during the time period following the regulatory approval but before the full diffusion and uptake of the product, i.e., early in the commercial lifecycle.



## PBRSA—Working Definition (2)

3. The price and/or revenue are either linked *explicitly* by formula to the outcome of this program of data collection (at a population level or at an individual patient level) or *implicitly* through an option to renegotiate coverage, price, and revenue later.

The arrangements are primarily concerned with reducing uncertainty about expected health outcomes (i.e., better estimates of real-world effectiveness): this can affect both uncertainty about the overall cost-effectiveness and about the utilization of the product (i.e., will the “right” numbers and types of patients get the product?).



## PBRSA—Working Definition (3)

4. The uncertainty may be about:
- the expected outcomes of the treated population as compared to those with the use of an alternative treatment path;
  - the size and value of cost offsets, such as fewer hospital visits;
  - the proportion of patients who will respond. i.e., achieve a pre-set (minimum) outcome or surrogate marker;
  - whether the patients treated are the “right” ones, i.e., matching the levels of cost-effectiveness identified in the evidence currently available?



## PBRSA—Working Definition (4)

- These arrangements provide a different distribution of risk as between the payer and the manufacturer than conventional arrangements.
- They exclude arrangements that are primarily intended to be disguised price discounts. However, a number of the operational aspects that underpin a successful PBRSA may apply to such schemes.



## Thank You!



For more information on the ISPOR PBRSA TF:

<http://www.ispor.org/Taskforces/performance-based-risk-sharing-arrangements.asp>

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