REES

Réseau d'Évaluation en Économie de la Santé

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Performance-Based Risk-Sharing Arrangements – Good Practices for Design, Implementation and Evaluation

An ISPOR Task Force Report

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General Comments :

1. Definition

The definition of the program-based risk-sharing agreement (PBRSA) is well tailored, especially because it clearly notes the difference between financial-based (budget capping, utilization capping, discount, etc.) and outcome-based arrangements. It is emphasized that PBRSA is a data collection program following marketing authorizations, linked to outcome of the program. From this perspective, the authors' proposal to use evidence collection to check "whether the medical product is used in the right patient" (l. 54) may be ambiguous, as it might provoke the implementation of observational studies without a comparator.

2. Taxonomy

The distinction between PBRSA performance-linked reimbursement at a patient level and PBRSA with evidence development is well thought. The former's goal is to adjust outcomes and prices, such as to obtain value for money. The latter's is to link reimbursement to prospective data collection. France gave a good example of such a thing with its program of "Stratégie Thérapeutiques Innovantes et Coûteuses" (STIC), which has been introduced in 2002, in which onerous drugs first and then after 2005 onerous devices are funded by public funds, provided that an economic protocol is submitted and validated by public authorities. However in such a scheme neither ex-ante nor ex-post reimbursements are specified.

3. Key good practice questions

The document's authors are too vague in their recommendations on evidence collection (Q2), maybe as a rule of thumb, as the subject has already been tackled in many ISPOR reports, but it would be good to remind us all that there is no evaluation without a comparator, whatever the design of the randomised clinical trial or observational study might be (cf. Rubin's Canonical Theory). For observational studies, study plans or statistical plans could be suggested : matched groups based on the propensity score, stratification, double differences, regression on discontinuity, etc.

Lines 103 to 105, the authors make reference to the temporary authorization scheme in France, it would be worth noting that if ATU are conducted within RCTs, the price of the product used within the ATU is left to the manufacturer's choice.

4. Remarks on box 3 : France

It is a good description of the ambiguities surrounding the post-launch of observational studies which may be required by the TC, focusing on "the use of a new product in real life". Those studies have nothing to do with performance but are used to adjust the price-volume requirements. As

emphasized in the text it is not clear if the results of such studies have affected reassessement. The explanation of this is quite clear, most often such studies do not use any comparator, so the relative effectiveness cannot be assessed, therefore the French public authorities are asking for studies that they readily know will prove unexploitable. But this does not hurt anyone.

Paris, may the 8th, 2012.

Professor Robert Launois.