Author's response to reviews

Title: Review of risk sharing schemes for pharmaceuticals: considerations, critical evaluation and recommendations for European payers

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Author's response to reviews: see over
Addressing Reviewer comments

A) Reviewer: L M.A Braakman

1) The introduction doesn't help me in getting familiar with the topic of 'risk sharing schemes'. Although I do have experience as a policy maker within the field of health economics, the authors couldn’t make clear to me what they exactly mean with this term.

Comment

We thank the reviewer for this comment, and we hope we have now made this clear in the section on definitions. Prior to this, we have re-emphasised that a wide variety of terms have been used to discuss risk sharing in the past and this terminology confusion needs to be addressed by those actually evaluating and implementing such schemes in practice rather than people working directly or indirectly for pharmaceutical companies.

2) The topic as well as the scope of the study is not explained clear enough. The title suggests this is a review of European Agencies. However, when checking table 1, it does not give a complete overview of all European countries. However, Canada, USA and Australia are included.

Comment

We thank the reviewer for this comment and hope we have now fully clarified the scope of the paper in the revised paper. We acknowledge that this review mainly covers Europe. However, we have provided details of schemes in Australia, Canada and Europe to emphasise to the readers the many risk sharing schemes that do exist around the world. We also explain in the methodology that this paper is NOT a comprehensive review of all risk sharing schemes in operation as others have recently done this. However, the schemes documented are illustrations of each type from different countries especially European countries.

3) Furthermore, I don't have a clue of the method the authors have chosen to compare the healthcare systems of the different countries. The methodology is not transparent. Therefore, the value of the results and discussion section is doubtful.

Comment

We hope we have now made clear that this paper is not a paper comparing different healthcare systems. However, it does suggest a logical working definition going forward, illustrates schemes under each title, and suggests the characteristics of future schemes having reviewed areas of concern.

B) Reviewer: Libby Roughead

1) I think this paper addresses a really important topic in an area where there has been very little work published. I suspect this paper has the potential to add significantly to the literature in this area. However, in its current presentation I think the paper is perhaps too ambitious and with insufficient structure to achieve this purpose.

Comment

We thank the reviewer for these positive comments, and hope the revised paper addresses previous concerns.

2) My main concerns are with the methods section and presentation of results. While the medline search is appropriately described. The description of the search of the grey literature is poor and requires more description. I think it insufficient to say you just went to websites you knew of.
What is most lacking in the methods, and thus in the results though, is how the information was then structured and synthesized. I think it would really help this paper if you provided definitions of your categories in your methods section - what is the definition of PVA used, what about caps and what about outcomes.

Comment

We thank the reviewer for these comments and hopefully we have addressed the concerns in the revised methods section. We have also extended the definitions section to address identified concerns.

3) Then I think you should indicate how you decided which examples fitted each definition (e.g. one author only, multiple authors independently). I say this as I am most familiar with the Australian system and none of the examples presented in table 1 for Australia are examples of what is considered in Australia a price volume agreement. Price reduction at the time of listing is not considered a price volume agreement - but rather the negotiated cost-effective price. Price volume agreements in Australia, are related to a reduction in the price once the volume exceeds the expected maximum market for the cost-effective indication (meaning use has extended into the market where the product is not cost effective at that price). I am not sure where the information came from that companies typically provide free drugs on our section 100 scheme - I don't believe this to be true (the example presented is rare, not "typical". In the Abacavir example, it was the company only asking the PBAC to pay for 2 of 3 supplies, not that that PBAC would only fund 2 of 3 and this comes about because of companies not wanting to drop global floor prices - I don't think this to be a true price volume agreement - hence the importance of definitions.

Comment

We thank the reviewer for this comment and have adjusted the tables to more clearly the different PVA schemes from the Abacavir ‘patient access’ example. The authors of this paper have a contract with the Australian Government Department of Health and Ageing to review submissions to the PBAC. In addition, one of the co-authors was Chair of the Economics Sub-Committee of the PBAC and member of PBAC from 1995–2000. Hopefully, this fully addresses the reviewer’s concerns about the authorship of this paper.

4) The lack of definitions makes it difficult to know what is truly being compared across countries and I also think it important in the tables to identify when something applies to the whole country (such as Australia with one national system) as opposed to parts of a system e.g. the US.

Comment

We thank the reviewer for this comment. As previously stated, we have provided details of the schemes as examples which can apply whether you have national or regional systems. Consequently, we have not taken this further as it only principally applies to schemes quoted for Spain, Sweden and the US.

5) The headings in the results and discussion section should match the aims and I can’t see this to be the case. Throughout the results and discussion there are phrases such as “There are concerns” - these need to be identified much more rigorously - consumer concerns, industry concerns, payer concerns - is it just an opinion of a member of the payer group or is it a formal statement from the payer. Similarly, the criticisms - stakeholder criticisms?? Is this a valid way to assess a risk sharing scheme? What about health and economic outcomes?

Comment

We thank the reviewer for these comments and have tightened the language in the revised submission. We hope this will now satisfy the journal.

6) Also in the results section are "we propose" "we believe", bits of results and case studies. I think if you untangle this to results first, case studies as illustrations if you wish, but then linked back to
overall synthesis, with recommendations in the discussion, the structure would be improved. Also in the results are statements such as “as discussed” which actually refer back to information in the tables, not information which is appearing earlier in the text. This makes it very difficult for the reader to follow. The authors also make recommendations, but it is hard to see, upon which evidence this is based and it would be stronger if this could be made clear.

Comment

We thank the reviewer for the comments and have tightened the language accordingly in the results section. We have also aligned the discussion to start with the financial based schemes, as well as again tighten the language to ease the flow. We have also moved the Tables documenting the various schemes to the end of the paper as an Appendix to further ease the flow so the reader does not become bogged down reading each scheme.

7) I do think this is a really important and there is a lot of useful information gathered, but I think it currently not if a format that provides enough rigour to be helpful to the debate.

Comment

We thank the reviewer for the positive comments and hope our revisions help address previous concerns.

C) Reviewer: Paul V Grootendorst

1) Traditionally, drug plans entered into relatively simple contracts with drug manufacturers. The drug plan reimbursed the manufacturer at a fixed rate per unit dispensed to plan beneficiary. Increasingly, these simple contracts are being supplanted by more elaborate contracts. These more elaborate contracts include so-called “price-volume” contracts in which the price paid per unit varies with the number of units dispensed. One example is a “hard budget cap” scheme wherein the price paid per unit drops to zero once price times volume exceeds some pre-specified amount. Another example of a more elaborate contract is the so-called “pay for performance” (PFP) scheme wherein the price paid per unit depends on the clinical status of the beneficiary receiving the drug. Typically prices are higher if the patient realizes some clinical improvement after taking the drug. The present paper enumerates some of these contracts entered into by pharmaceutical firms and private and public drug plans in the EU, Canada, and the USA.

Comment

We thank the reviewer for summarising the different types of contract in existence – hence the need to define a workable and logical definition from a payer’s perspective going forward.

2) It claims that such non-standard contracts must be used into the future in the EU so as to “enable the continued provision of comprehensive and equitable healthcare.” It provides some commentary on the success of these more elaborate contracts and provides some guidance to policy makers considering their use.

Comment

We have documented that there has been an increase in risk sharing schemes in recent years, and provide some rationale for this. However, we have made clear that we have severe concerns with many schemes, which need to be addressed before any further expansion should be considered by payers in the future.

3) My overall impression of the paper is that a suitably revised version could be a useful contribution to the literature. However, as it currently reads, the paper is too disorganized. It requires some focus and structure. Moreover, it needs to consider the effects that the various contractual schemes have on the incentives of drug developers in producing effective new therapies.

Comment
We thank the reviewer for these comments and hope we have addressed a number of these concerns in the revised paper. From a health authority perspective, we totally disagree that there should be further incentives for pharmaceutical manufacturers and quote one paper where one Pharmaceutical Company in one year spent more money on marketing that either the manufacturers of Pepsi Cola or Budweiser. Consequently, if anything the incentives are too generous for pharmaceutical companies putting health authorities under increasing pressure with the continued launch of new premium priced drugs often with very limited health gain versus existing standards.

4) The section of the paper “Definitions and legal status ” can be safely integrated into other sections.

Comment

We respectively disagree with this as we believe it is essential to devote a section to the terminology given the wide variety of terms used under the umbrella of 'risk sharing' which is emphasised by the reviewer in his initial remarks.

5) There is little value in the paragraph beginning “In some countries such as Poland, new laws are needed before risk sharing schemes can be fully enacted”.

Comment

We thank the reviewer for this, and have tightened this whole section in the revision.

6) The authors might consider the following structure: Why the non-standard contracts? This would position the contracts as providing advantages to both parties relative to standard contracts. Example: a PFP – a scheme wherein reimbursement is paid conditional on clinical success – can provide value for money to payers while allowing drug companies to demonstrate their drug’s value in ways that are not possible using clinical trial data. Desirable features of non-standard contracts between drug plans and drug companies. This would consist of a set of normative criteria, suitably justified, with which to evaluate the contracts. For instance, one criterion would be verifiability: it is possible for both parties to the contract to verify whether patient health outcomes have improved (in the case of performance based contracts). Another is credibility: there are mechanisms that ensure both sides to the party live up to their end of the bargain. What kinds of non-standard contracts have been struck between drug plans and drug companies? This is largely already covered in the tables and accompanying text. What are the predicted and actual outcomes of these contracts? What is the predicted success of these contracts (predicted according to the extent to which they meet the normative criteria?) What is known about actual success of these contracts in achieving outcomes?

Comment

We thank the reviewer for these comments and believe we have covered the rationale behind risk sharing schemes in the revised paper. In addition, we believe we have further stated and refined where we believe ‘risk sharing’ schemes could be considered by payers in the future and where they should be rejected. This may be different to pharmaceutical company agenda’s – but this should not be a major consideration from a health authority perspective especially given the limited health gain with most new products. We believe we have also further refined a set of clear criteria from a payers’ perspective going forward. This includes the instigation of independent clinical trials to verify patient outcomes to reduce bias from any company conducted studies. We have also emphasized that it can be difficult for companies to verify schemes especially where this involves access to patient data. However, this is a factor which is difficult to circumnavigate and one that must be accepted by pharmaceutical companies going forward.

Lastly, we make a plea about evaluating future risk sharing schemes especially for pharmaceuticals given the paucity of published evaluations. There have been more evaluations for other technologies, which we document in the revised paper.
D) Reviewer: Anna Birna Almarsdóttir

1) This manuscript was a very interesting read, well organised and thorough. I recommend it be published without any significant major Compulsory Revisions.

Comment
We thank the reviewer for her positive comments.

2) There are a couple of issues that can easily be dealt with: The difference between PVA and price caps is not entirely clear and a sentence or two to explain would be good to add on page 3.

Comment
We thank the reviewer for her comments and hope we have addressed this in the revised paper giving discrete examples.

3) Please explain what you mean by learning from procurement. I am not clear on how and what is to be learnt.

Comment
We have now removed this reference in view of the confusion this caused.

4) Minor Essential Revisions: Explain on p.10 what is meant by "the two different schemes" (middle of the page).

Comment
We thank the reviewer for her comments and have now addressed this in the revised paper.

5) Typos on p. 11: "... are not taken as recommended." and "... members who have previously been poor compliers..." (middle of the page). Write Primary Care Trusts (PCTs) instead of just the acronym.

Comment
We thank the reviewer for her comments and have addressed these in the revision.

Discretionary Revisions

6) Table 1: The authors suggest that this table can be put in an appendix. If this is done, then there needs to be text to explain broadly what the examples entail. I find all the tables very informative.

Comment
We believe we have addressed this with the inclusions of definitions, etc.