Author’s response to reviews

Title: Evidence-based decision-making for diagnostic and therapeutic methods: the changing landscape of assessment approaches in Germany

Authors:

Britta Olberg (britta.olberg@googlemail.com)

Sabine Fuchs (sabine.fuchs@tu-berlin.de)

Katja Matthias (katja.matthias@g-ba.de)

Alexandra Nolting (alexandra.nolting@g-ba.de)

Matthias Perleth (matthias.perleth@g-ba.de)

Reinhard Busse (rbusse@tu-berlin.de)

Version: 2 Date: 31 Aug 2017

Author’s response to reviews:

General comment to the editor: We have made significant structural changes in response to the reviewers’ comments. Does this have any effect on the category we were asked to submit our paper (i.e. ‘opinion’ paper)?

Reviewer #1:

General comments:

Comment Reviewer #1: I have read this article with interest and think that it has potential to make a relevant contribution to this journal. I am always keen to read about the decision-making processes of the Federal Joint Committee and am sure that there is international interest in the German approach to health services coverage. However, I have three main criticisms: The current version of the article is both too detailed and too unclear to be accessible for an international audience. The article is also unclear about its aims and argument, and would benefit from refocussing and reorganisation. The use of language also requires attention (see terminology).
Answer to comment of Reviewer #1: We rewrote, restructured and (partially) shortened the whole text in order to address all of the reviewers’ comments as far as possible. The content is now clearer, more focused and (hopefully) generally more understandable for an international audience.

Comment Reviewer #1: In its current form, the article presents much detail about the trajectory of health technology assessment in Germany, but it is not clear what the article contributes to the knowledge of readers outside the system which this journal aims to inform (especially in low and middle income countries). So the article should be clearer in answering the "so what?" question: Why do we need to know about these developments? What is interesting about them? Which larger body of research does this article contribute to? This would also help to set the paper apart from previous "histories" (e.g. Perleth et al. 2009) in addition to presenting an update on developments. The answer to these question will determine the aims and organisation of the paper, and this could lead to very different types of papers.

Answer to comment of Reviewer #1: We appreciate this remark and tried to address these aspects as far as possible in our revision of the paper. Especially, the new background chapter should be more concrete regarding the specific aims of this case study and what it contributes to the knowledge of readers outside the German system.

Comment Reviewer #1: The article is currently framed as a contribution to debates about evidence based medicine and health technology assessment, but these fields have moved on considerably over the last decades and are now much more diversified. I recommend that the authors tease out what is special about the German 'case' of institutionalising HTA in decision-making on publicly funded health service coverage, focus on these aspects in detail (while discarding other debates) and frame the paper accordingly. I understand that the article is laid out as a history of developments and not as a piece of empirical research, but even as a history it remains vague and uninteresting especially as the actors who have shaped these developments (e.g. as legislators, as members of the self-governance system) are conspicuously absent. 'Politics' feature occasionally, but political arguments are often clouded by the use of language, which implies actors but never mentions them. If this is about legislative changes over time (rather than, say, methodological changes to HTA) the article needs to say so upfront and focus on changes to the legislative framework, the dynamics that have informed them and the implications for the GBA.

Answer to comment of Reviewer #1: We thank the reviewer for this remark. By rewriting and restructuring (whole) parts of this paper, we tried to apply all suggestions as far as possible. This applies especially to a clearer description regarding the importance of Germany as a case sample and the focus/aim of this study.
Comment Reviewer #1: In several places, the article seems to measure the German approach on international EBM "best practice" (whatever this might mean). This should either be made more explicit, which than means that it has to be established first what "best practice" in this field looks like, or it may not be appropriate to make such normative statements. As it stands, other countries make a showing on the final page but the comparison does not inform the framing of the article.

Answer to comment of Reviewer #1: Based on the revisions made throughout the paper, we now addressed this remark as far as possible. During the revision of the discussion section, we removed the paragraph on the comparison to other countries.

Specific comments:

Background:

Comment Reviewer #1: The background should present a clearer aim of the article, which needs to reflect the aims and structure of the main text. An "outline" is too weak and "an outline of the current status and developments regarding" is not accurate (if anything it is the other way around). In other parts it is referred to as "review" (p3). However, I think it is essential that the "so what?" question is addressed - what is relevant to know about the German system for an international audience?

Answer to comment of Reviewer #1: The whole background chapter was restructured and rewritten aiming at presenting a clearer description of the aims and the ‘so what’ question of the study. The abstract was also adapted.

Comment Reviewer #1: As mentioned above, the paper could draw on a more specific literature focusing on the role of HTA in decisions on health service coverage in publicly funded health care systems and specific debates within this field. The broad focus on EBM feels inappropriate and outdated, unless it is used for something more specific such as comparing the German approach to EBM "standards" that have informed international best practice (not sure though whether there is necessarily consensus). This is likely to produce a less comfortable paper for authors who are associated with the GBA.

Answer to comment of Reviewer #1: We modified the background chapter as well as the sub-chapters in the main body of the text in order to improve the structure and clarify the content as a
whole. This also includes new/more literature on the role of HTA in policy decision-making in different countries.

Comment Reviewer #1: The discussion of medical devises and European activities would be interesting if it would resonate more strongly with the rest of the paper, but it only comes up towards the very end of the paper.

Answer to comment of Reviewer #1: Based on the revisions made throughout the paper, we tried to address this criticism as far as possible.

Comment Reviewer #1: P2, lines 50-52: The second part of the sentence does not follow from the first.

Answer to comment of Reviewer #1: Because of the revision of the whole background section, this content became obsolete.

Methods:

Comment Reviewer #1: The methods section is weak, which is probably owed to the fact that the article is written almost as a legal history. This section should provide more detail on how documents were obtained and how they were analysed. Policy documents etc. do not constitute "relevant literature" (see abstract). They are data sources. The problem with this history is that such "data" is very formalistic (for want of a better word), especially if these are official regulatory and legal documents, which allow little insight into the dynamics that have motivated the changes and developments that the article is interested in. This is a methodological problem of such analyses, hence the importance to use additional data sources (e.g. interviews).

Answer to comment of Reviewer #1: We tried to address the criticism of the reviewers as far as possible. As suggested by reviewer #2, we created a separate methods section which entails the information originally presented at the end of the (old) background chapter. Moreover, we are more specific/added further information regarding for example the time frame, type of the documents, etc.
Main body:

Comment Reviewer #1: The main body of the text presents the development of HTA informed decision-making in Germany. These sections would work better if more attention would be given to actors and to those aspects that are most relevant to an international audience (i.e. the "story" needs a clearer focus). This problem affects both the aims/focus on the article and the style of writing. For example, a sentence like "The idea of technology assessment was initially discussed by the technology assessment office of the parliament." (p4) raises more questions than it answers: When was initially? What is the technology assessment office of the parliament? What do they have to do with HTA or health insurance coverage? If they were a key agenda setter, have they played any further role in the development of HTA in Germany?

Answer to comment of Reviewer #1: We thank the reviewer for this comment. In order to address the suggestion as far as possible, we partially rewrote and restructured the main body of the text. Now, more attention is given to the most relevant actors (within the G-BA) involved in the German decision-making process for diagnostic and therapeutic methods. In addition, other sections, which are affected by these changes, were aligned accordingly (e.g. discussion section).

Comment Reviewer #1: In places, information needs to come earlier, e.g. p 5 "such as the predecessors of the G-BA" (who are they?) Followed by "In 2004, ..., the former Federal Standing Committee ... " in the next paragraph. OK - Are they the predecessors?

Answer to comment of Reviewer #1: Based on the general revisions made throughout the paper, we hope that this criticism has been addressed as well.

Comment Reviewer #1: The GBA is referred to in different ways e.g. "the highest decision-making body of the joint self-government of ..." (p2) and "the supreme decision-making body" (p5) (I would avoid "supreme" in relation to public administration, which sounds like North Korea). If in doubt, use the wording that the organisation uses for itself.

Answer to comment of Reviewer #1: We thank the reviewer for this remark. To be consistent within this article, we continuously refer to the G-BA as "the highest decision-making body of the joint self-government of physicians, dentists, hospitals and health insurance funds in Germany".
Comment Reviewer #1: I would separate health technology assessment and coverage decisions as two (related but) separate processes. "Benefits assessment" (p6) seems to conflate both. In my view, HTA is the EBM informed process, while decision making is as corporatist (and therefore political) as it always was give and take a few important changes such as changes to the voting rights, involvement of patient organisations etc. These processes need to be separated out.

Answer to comment of Reviewer #1: We thank the reviewer for this comment. The HTA process and the final coverage decision are not that easy to separate within the German system. The reviewer is right that these processes are related but they will happen consecutively (i.e. first an HTA is performed, followed by the coverage decision, based on that assessment). Nevertheless, the final decisions may turn out differently. During the revision of the main body of the text, we clarified this issue.

Comment Reviewer #1: P 6 It is a peculiarity of German policy-making that terms such as "methods" as health technologies are defined by the courts. This may require more background information.

Answer to comment of Reviewer #1: We added more details aiming at clarifying the content. Additionally, the definition of diagnostic and therapeutic methods was placed earlier in the paper (i.e. in the new methods section).

Comment Reviewer #1: "Wirtschaftlich" does not translate into "cost-effective". It is a legal term (perhaps expressing an ambition), not underpinned by any method of assessment (parliament has decided against cost-effectiveness analysis as a method), except perhaps some cost impact analysis (although this pans out differently for pharmaceuticals).

Answer to comment of Reviewer #1: The whole paragraph has been rewritten. The term cost-effective is not used anymore.

Comment Reviewer #1: "Principles of EBM" - what are they? (p7). If they are key to the argument they should be brought to the fore and discussed in the beginning. There is also mention of "the methods of EBM" (p12) - what are those?
Answer to comment of Reviewer #1: We clarified the content by adding an explanatory sentence. In addition, we exclusively use this term throughout the text in order to be more consistent.

Comment Reviewer #1: P7 "Methods which are not necessary or ineffective" - this is legal text and should be treated as such. This is important given that what is seen as "not necessary" is often controversial.

Answer to comment of Reviewer #1: The reviewer is right. We changed the sentences to: ‘Methods, which are ‘not necessary or inefficient’, should not be included in the SHI benefit basket [19]. However, as to what constitutes necessity is sometimes controversial between the actors in the G-BA.’

Comment Reviewer #1: "Eminence-based medicine" - this is a German in-joke that is not necessarily understood by an international audience (I have tried it and failed). The way it is presented seems to suggest that it is used in official parlance.

Answer to comment of Reviewer #1: We deleted the term 'eminence-based medicine’ and rephrased the sentence in order to be more clear.

Comment Reviewer #1: P9 I disagree that the processes before 1997 were "opaque" compared to today as this seems to imply that processes now are "transparent". This may be accurate with regard to the use of HTA as an assessment tool but it underplays the lack of transparency of the decision-making process between the corporatist actors who form the GBA.

Answer to comment of Reviewer #1: We modified the sentence in order to be more concrete: ‘Despite these developments, until 1997, the decision-making process of the Federal Standing Committee of Physicians and Sickness Funds was less transparent, as only the decisions themselves were published, but not how they were formed (i.e. through published rules of procedure).’
Comment Reviewer #1: P9 "Its work was being increasingly denounced as arbitrary and incoherent" requires a reference. By whom?

Answer to comment of Reviewer #1: We added a reference.

Comment Reviewer #1: P11 "Innovations in health care find their way" - this is too unspecific and not well worded.

Answer to comment of Reviewer #1: We modified the sentence as follows in order to be more specific: "Most new diagnostic and therapeutic methods in Germany are first introduced in the inpatient sector, which…”

Comment Reviewer #1: "This is justified by the (political) assumption that" - whose assumption specifically? How comes they have influence over the legislative framework? Why does this make the decision "justified”?

Answer to comment of Reviewer #1: We modified the content as follows: "The primary intention by the legislator of this rule is to ensure that insured patients are rapidly able to take advantage of innovative methods of treatment. Therefore, in the inpatient sector innovations in diagnostic and therapeutic methods find conditions that enable their rapid application in clinical practice [28].”

Comment Reviewer #1: Table "Approach not existent" - not clear what this means.

Answer to comment of Reviewer #1: We changed “Approach not existent” to “No approach for this situation existed”.

Comment Reviewer #1: P12 Some parts of this paragraph may work better in the past tense.

Answer to comment of Reviewer #1: Suggestion applied as far as possible.
Comment Reviewer #1: P13 "reflect the beginning of a shift towards improvement of evidence-based decision-making"/ "apparent room for improvement" - this is a normative judgement that seems out of context (unless the paper is framed differently and the German approach is compared to a standard, but not sure this is the intention, it would make a much more critical paper).

Answer to comment of Reviewer #1: We modified the content in order to prevent using such normative formulations.

Comment Reviewer #1: "This can be explained by remaining inconsistencies between the two health care sectors in Germany" - Does it? Not sure the preceding paragraphs suggests that this is the reason. Are there alternative explanations?

Answer to comment of Reviewer #1: We tried to clarify the content as far as possible.

Comment Reviewer #1: "Health policy-making has partially responded" - Policy-making is not an actor, it therefore cannot respond.

Answer to comment of Reviewer #1: This is correct. We changed ‘’Health policy-making’’ to ‘’Health policy-makers’’.

Comment Reviewer #1: "to the criticism" - whose criticism?

Answer to comment of Reviewer #1: We added more details.

Comment Reviewer #1: P 16 - This requirement can be understood as a reversal of the burden of proof." - If this is about comparing the German system, does this mean this is a retrograde step? How does this relate to notions of EBM (if at all)?

Answer to comment of Reviewer #1: The reviewer is right. We addressed this remark by adding a sentence accordingly.
Comment Reviewer #1: "market approval process" - probably better as "market authorisation", which is the official term used by EMA.

Answer to comment of Reviewer #1: Suggestion applied.

Comment Reviewer #1: "Consequently, benefit is simply assumed" - by whom?

Answer to comment of Reviewer #1: We modified the content in order to be more specific. During the revision, this sentence became obsolete and was deleted.

Comment Reviewer #1: "these remaining deficiencies" - again, I would be careful with such normative claims. It also sounds as if there are only a few "deficiencies" remaining, but I am not sure this is consensus.

Answer to comment of Reviewer #1: We modified the sentence in order to be more concrete: "'(…) the German legislature aimed at correcting the deficiencies of the existing regulations by introducing (…)’”.

Comment Reviewer #1: "A new statutory regulation" - is this different from law?

Answer to comment of Reviewer #1: We replaced “statutory regulation” with “stipulation” in order to be more clear and consistent with other parts of the text.

Comment Reviewer #1: "interrelates" - not a good word in this context. Perhaps "amends"?

Answer to comment of Reviewer #1: Suggestion applied.
Comment Reviewer #1: P17 "methods based on high risk MDs" - this is the first time that medical devices are mentioned in the empirical part of the paper. This seems very much as an add-on which is not what I expected given its prominence in the introduction.

Answer to comment of Reviewer #1: Based on the revision of the whole manuscript, this criticism has been addressed.

Comment Reviewer #1: "Coalition agreement"- this (I think) is the first time party politics is mentioned. If this is a relevant driver of developments, which I think it is given the importance of changes to the legislative framework to the work of the GBA, reference should be made to politics more consistently.

Answer to comment of Reviewer #1: Suggestion applied as far as possible. We rewrote and restructured the main body of the text in order to name the most important actors involved in the decision-making process of diagnostic and therapeutic methods in Germany (please also consider the answer to the reviewers’ first comment).

Discussion:

Comment Reviewer #1: This discussion is not a discussion. It presents new information which it should not. It should relate back to the literature introduced earlier (i.e. international discussions on using HTA to inform health service coverage decisions in publicly funded health systems). It is striking that the discussion focuses on medical devices. Perhaps this should be a separate "results"/history chapter?

Answer to comment of Reviewer #1: We rewrote and restructured (by adding sub-headings) the whole discussion section in order to address the reviewers’ criticism. Additionally, we clarified in the background and methods section that the focus of this case study lies on the HTA informed decision-making process for diagnostic and therapeutic methods. Decision-making processes in the context of other health technologies (e.g. pharmaceuticals) are not within the scope of this study.
Comment Reviewer #1: P17 "increasing concerns" - by whom?

Answer to comment of Reviewer #1: Based on the revision of the discussion section, this formulation became obsolete.

Comment Reviewer #1: P18 "a fairly consistent development during the last 20 years" - Not sure I agree. There is certainly movement, but it does not always go in the same direction. It is telling that cost-effectiveness is absent in this paper. How about the argument of the "reverse burden of proof".

Answer to comment of Reviewer #1: We addressed this criticism by modifying the content as follows: "However, from 1998 onward and in particular after 2012, the importance of evidence utilization (HTA) in legitimizing decisions, despite some conflicting steps (e.g. the two-third majority for the exclusion of a method), increased overall."(…)

Comment Reviewer #1: P19 "This is alarming, as patients might be exposed to a potentially harmful method" - True, but the discussion of potential harms needs to be introduced much earlier. It seems only an afterthought here, but as far as I am concerned harm avoidance is part of the entire rationale of EBM.

Answer to comment of Reviewer #1: We thank the reviewer for this comment and addressed this issue earlier in the text.

Comment Reviewer #1: P20 "innovation-friendly principle of 'permission unless explicitly banned" - a politically driven euphemism? This argument should be much more critically made. This is the territory of political argument, not a matter of fact.

Answer to comment of Reviewer #1: In order to follow the reviewer’s suggestion, we modified the sentence as follows: ‘In addition, the controversial principle of the “Verbotsvorbehalt” (in accordance with § 137c SGB V) in hospitals will be partly replaced by these processes.’
Comment Reviewer #1: Here, examples of other countries are mentioned but only in a cursory fashion. It is important to bring out the international relevance of the German experience much more clearly.

Answer to comment of Reviewer #1: The reviewer is right. We rewrote the discussion section. Within this process, the examples from other countries became obsolete.

Comment Reviewer #1: "some even for longer time than in Germany" - the "even" is not appropriate for an international journal.

Answer to comment of Reviewer #1: Based on the revision of the whole discussion section, we deleted the paragraph regarding the comparison to other countries. Therefore, this formulation has become obsolete as well.

Comment Reviewer #1: Not sure the differences between Germany and other countries is adequately captured. Does the "forth hurdle" not usually relate to cost-effectiveness analysis as a criterion for reimbursement decisions? If this is the difference (which I think it is) this has to be spelled out.

Answer to comment of Reviewer #1: The reviewer is right. Based on the revision of the whole section, we removed the paragraph regarding the comparison to other countries. In the process, the formulation of the ‘fourth hurdle’ also became obsolete.

Comment Reviewer #1: P20 "Considerable inconsistencies between in-and outpatient care settings" - this only warrants mentioning at the top of the conclusion if this is the main focus of the paper.

Answer to comment of Reviewer #1: We rewrote the conclusions section. Please see changes made.
Comment Reviewer #1: "learning system" - yes, but some lessons have not been learned or there was a political dynamic in place that did not allow for changes to be made. So this needs to be qualified.

Answer to comment of Reviewer #1: We thank the reviewer for this remark. We added more details in order to clarify the content.

Terminology:

Comment Reviewer #1: The terminology used also needs revisiting for a publication aimed at an international audience. It also should be more consistent.

Answer to comment of Reviewer #1: We thank the reviewer for this remark. We followed the specific suggestions regarding terminology given by the reviewer and hope that the general changes in this revision of the article also enhanced the consistency regarding the terms used.

Comment Reviewer #1: The German "Methoden" does not translate easily into English as "methods", which are typically used for research methods and the like. "Health services" or "health technologies" are more common terms.

Answer to comment of Reviewer #1: We thank the reviewer for this remark. We decided to stick with the term ‘methods’ as it has been established over time and throughout various legal decisions.

Comment Reviewer #1: Some wording sounds a little too directly translated, e.g. "as a decisive basis" (p2) - "to inform decisions" would be better.

Answer to comment of Reviewer #1: Suggestion applied.
Comment Reviewer #1: "Direct corrective action" (p10) does not translate into meaningful English ("corrective" sounds like the penal system to me). Would "commissioning evaluation" or "health services research" be appropriate perhaps?

Answer to comment of Reviewer #1: Suggestion applied and changed to “no possibility to commission evaluation”.

Comment Reviewer #1: The word "ban reservation" is not a good translation of "Verbotsvorbehalt", which is probably better to be paraphrased. It is referred to as "prohibition right" (p 12) elsewhere, but this does not make it clearer.

Answer to comment of Reviewer #1: We thank the reviewer for this remark. As there is no official translation for this term, we decided to use the original German expression consistently throughout the article, with an explanation when the term was used for the first time.

Comment Reviewer #1: "Operator effects" (p3) needs an explanation. "Testing directive" is unclear.

Answer to comment of Reviewer #1: The paragraph including the term “Operator effects” was deleted due to the general revision of the background chapter. We now present more information in the subsection ‘Introduction of the coverage with evidence development (CED) reform (§ 137e SGB V)’ in order to be more concrete about the assessment of the potential and the testing directive.

Comment Reviewer #1: Some wording is inappropriately normative (e.g. "a very useful concept and tool" (p2) - better "an established tool").

Answer to comment of Reviewer #1: Correction applied.
Comment Reviewer #1: Some phrases that are common in German HTA parlance may need explanation (e.g. "patient-oriented decisions").

Answer to comment of Reviewer #1: We rephrased the paragraph in order to be more clear.

Comment Reviewer #1: "Proprietary digital databases" needs an explanation, especially as they seem to comprise both organisational information management systems and profit oriented database outlets.

Answer to comment of Reviewer #1: Based on the revision of the abstract, this wording became obsolete.

Comment Reviewer #1: "Controversial debate" (p3) is a tautology.

Answer to comment of Reviewer #1: We removed "controversial".

Comment Reviewer #1: "No proven evidence" is also a tautology (p18).

Answer to comment of Reviewer #1: We modified the sentence and used ,,insufficient or even no evidence”.

Comment Reviewer #1: "Evidence-based technology assessment" - is this different from "health technology assessment"?

Answer to comment of Reviewer #1: We thank the reviewer for this remark and use the term “HTA” instead.
Reviewer #2:

Comment Reviewer #2: You have given a very clear and informative overview on the development of use of evidence-based decision-making in Germany. I suggest to include a separate chapter on methods, not to put the relevant information only at the end of the background chapter. In this methods section you should describe in a bit more detail in which data bases, within which time periods and when you have searched.

Answer to comment of Reviewer #2: Suggestion applied as far as possible. We created a new methods section and added further details.

Comment Reviewer #2: In the discussion/ conclusion section a short passage on possible limitations of your study (had you access to all information available? etc.) should be included.

Answer to comment of Reviewer #2: Suggestions applied as far as possible.