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Health benefit assessment of pharmaceuticals: An international comparison of decisions from Germany, England, Scotland and Australia

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ABSTRACT

Background: Little is known on the performance of the newly introduced health benefit assessment process, AMNOG, in Germany compared to other health technology assessment agencies.
Objective: We analysed whether decisions of the German Federal Joint Committee (FJC) deviate from decisions of the UK National Institute for Health and Care Excellence (NICE), the Scottish Medicines Consortium (SMC) and the Australian Pharmaceutical Benefits Advisory Committee (PBAC).
Methods: We analysed decisions made for comparable patient subgroups by the four agencies between 2011 and 2014. First, decisions were compared (a) by their final outcome, i.e. whether a health benefit was identified, and (b) by the agencies' judgement on comparative effectiveness. Subsequently, we partially explored reasons for differences between HTA agencies.
Results: From the 192 FJC decisions, we identified 55 that overlapped with NICE, 166 with SMC and 119 with PBAC. FJC agreed with NICE in 40% in final outcome (Cohen's Kappa = −0.13). Similar results were obtained for FJC and SMC (47.6%, kappa = 0.03) and FJC and PBAC (48.7%, kappa = 0.07). Agreement increased when comparing judgements based on comparative effectiveness only. However, the FJC's final decision was positive only in 43.6%, 39.2% and 44.5% of the patient subgroups, as opposed to 74.5% (NICE), 68.7% (SMC), and 68.9% (PBAC), respectively.
Conclusion: We show that the FJC – an agency relatively new in structurally assessing the health benefit of pharmaceuticals – deviates considerably in decisions compared to other HTA agencies. Our study also reveals that the FJC tends to appraise stricter than NICE.

1. Introduction

Fourth-hurdle decision making helps to decide on a new pharmaceutical's coverage and reimbursement within a health care system. It is called 'fourth-hurdle' because the pharmaceutical has already passed three hurdles to achieve market authorization thereby demonstrating its safety, efficacy and quality [1,2]. Given the need to allocate

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scarce resources and to contain pharmaceutical expenditure, many countries have established fourth-hurdle decision making to assess and appraise technologies over the last two decades [3]. Among them are Australia, Belgium, Canada, England and Wales, Scotland, Sweden and the Netherlands [4]. The general aim is to assess the trade-off between health benefit and a pharmaceutical's cost. Despite being Europe's largest pharmaceutical market in terms of sales volume, Germany was a late-mover to implement the fourth hurdle in January 2011, by the Pharmaceutical Market Restructuring Act (AMNOG).

Within three months after market launch, all newly introduced pharmaceuticals are evaluated based on their added benefit over a comparator, the so-called Early Benefit Assessment (EBA) [5]. By law, manufacturers are obliged to submit a dossier to the Federal Joint Committee (FJC). Within six months after submission, the FJC performs the appraisal. Whilst the final decision is with the FJC, the Institute for Quality and Efficiency in Health Care (IQWiG) is, by convention, commissioned for a preliminary assessment. While IQWiG assesses the evidence submitted by the manufacturer in the first place, FJC is responsible for the final decision after a separate assessment of the evidence. It has been shown that FJC tends to soften IQWiG's decisions [6]. If an added benefit is approved by FJC, in a separate stage, the manufacturer and the Federal Association of Sickness Funds negotiate a price within another six months. Pharmaceuticals that do not show an added benefit become subject to reference pricing or other reimbursement restrictions.

To date, evidence on the German system focusses on discussions on the benefits and limitations of the AMNOG reform itself, the outcomes of the first wave of EBAs and the agreement between manufacturers, IQWiG and FJC [6–13]. However, little is known about how the FJC's judgements compare to other health technology assessment (HTA) agencies. First international comparisons have revealed differences in the process and provided qualitative overviews of decisions by therapeutic areas and prices [14,15]. Other studies examine consideration of indirect comparisons [10] or quality of life [16] in appraisals. This is of relevance to both the pharmaceutical industry and health policy makers.

While pharmaceutical product development aims to cover multiple health care markets, regulation ideally follows country specific preferences. This is why there are varying preferences towards the process and methods of evaluating new pharmaceuticals [17]. For this reason, the institutions that have emerged share some common features, but also differ in others. For the final decision, important criteria in many systems are the appraisal of comparative effectiveness, i.e. the appraisal of '*clinical information on the relative merits or outcomes of one intervention in comparison to one or more others*' [18] and, cost-effectiveness that analyses a substance's benefits in face of its cost. While cost-effectiveness is not considered in the FJC process, it is a common criterion of nearly all HTA agencies in the appraisal process. Comparative research thus helps (a) pointing out areas of disagreement between agencies when performing the same or similar tasks, (b) identifying and explaining drivers for deviation in outcomes and (c) improving decision-making processes.

Previous research has analysed the fourth hurdle through various means [19,20]. First quantitative approaches have focussed on the final appraisal, i.e. the resulting decision that may rest on varying criteria across HTA agencies and the appraisal of comparative effectiveness [21,22]. Qualitative approaches have explored possible reasons for variations in decisions by variation in the decision-making criteria and the reasons for differences in HTA including the varying interpretation in underlying uncertainty of the evidence [23]. Such approaches typically cover a smaller sample of decisions and specific product categories that allows in-depth analysis of the interpretation of available evidence by all types of sub-criteria and including the full complexity of decision-making. In this study, we focus on the final decision and the assessment of comparative effectiveness as these outcomes determine the degree of implementation in the health system after the decision and constitute what is perceived by stakeholders first.

The objective of this study was thus to compare the decisions of the German FJC with three other HTA agencies. We chose the English National Institute for Health and Care Excellence (NICE), the Scottish Medicines Consortium (SMC), and the Australian Pharmaceutical Benefits Advisory Committee (PBAC) as comparator HTA agencies. As a first step, we analysed the decisions made jointly by the FJC and the other agencies between 2011 and 2014. Finally, we partially explored drivers for deviances in outcome.

2. Comparator agencies

Evaluations of benefit in all four agencies are conducted in two stages, as separate institutions for assessment and appraisal are involved. FJC, NICE and PBAC appraisals are comprehensive, while SMC conducts a 'rapid early review' [24]. The trigger for the benefit assessment process differs: FJC and SMC appraise all newly licensed pharmaceuticals, PBAC requires manufacturers to actively seek reimbursement. NICE reviews all cancer drugs and most new indications or new entities as it sees fit. However, there is no formal requirement to review new drugs/indications to receive market access. This also implies differences in the timing of the assessment. FJC, PBAC and SMC appraise new entities early in a drug's post-development life cycle, while the time frame for NICE appraisals may vary. Furthermore, the consequences for pharmaceuticals' pricing and reimbursement differ. A negative decision by NICE, SMC and PBAC will exclude a drug from reimbursement. This may base on unfavourable comparative effectiveness or, if health benefits are present, cost-effectiveness. A negative decision of the FJC that solely rests on considerations of comparative effectiveness will 'only' impact reimbursement prices. Thus, the results from the appraisal of comparative effectiveness have differing consequences.

With respect to the type of evidence taken into account in the decision-making process, all agencies use clinical evidence for their appraisals. While the FJC's assessment is totally limited to clinical evidence and only evaluates comparative effectiveness [6], NICE, SMC and PBAC follow