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# Managed entry agreements for pharmaceuticals in Australia

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ABSTRACT

In Australia, a number of managed entry agreements have been developed to enable national coverage of new medicines. Non-outcome based agreements are usually pricing arrangements that involve price or volume rebate agreements. In February 2013, there were at least 71 special pricing arrangements in place, including 26 for medicines restricted to use in hospitals. Health outcome based agreements can be made at the individual or population level. At the individual level, there were 28 medicines funded subject to continuation rules involving documentation of adequate benefit within the individual; some of these medicines also had price agreements in place. At the population level, only one outcome-based agreement has been implemented so far, for bosentan, a medicine marketed for pulmonary hypertension. In May 2010, a memorandum of understanding signed between the Australian Government and Medicines Australia, the peak pharmaceutical industry organisation, included the possibility for industry to request consideration of a 'Managed Entry Scheme' as part of the funding submission process for medicines with high clinical needs. It includes the possibility of a randomised controlled trial (RCT)-based entry scheme. Although this form of managed entry has yet not been trialed in Australia, several 2012/2013 funding recommendations included requests by the decision making committee for further evidence development.



## 1. Introduction

The growing ageing population, the raising prevalence of chronic diseases and the high cost of new medicines are all factors contributing to rising pharmaceutical expenditures. The high prices requested by pharmaceutical companies for new medicines create further challenges. In 2012,eleven of the twelve cancer medicines approved by the US Food and Drug Administration were priced above US$ 100,000 per year [1]. The price of ivacaftor, a new medicine marketed in 2012 for a genetic disease, cystic fibrosis, reached US$373,000 per patient in the United States [2] and was recommended in Australia with an estimated cost of AU$60 million to $100 million in the fifth year of listing [3,4]. In 2013, the listing of pregabalin for chronic nerve pain will cost the Australian government more than AU$ 450 million over five years and those of three new cancer medicines, ipilimumab for advanced melanoma, abiraterone for advanced prostate cancer and oral vinorelbine for advanced breast cancer, will cost more than AU$430 million over four years [5].

Funding decisions by public or private insurance bodies are often difficult because of numerous uncertainties around the efficacy, effectiveness, safety or cost-effectiveness of new medicines [6]. This uncertainty can create cases where medicines receive a positive coverage decision, however, subsequent evidence indicates the medicine is found not to be as effective or as safe as

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expected and thus resources are wasted (i.e. opportunity costs) or patients harmed. Equally challenging is the negative coverage decision for a breakthrough medicine, which may prevent some patients from accessing the medicine. Moreover, public and private insurers often operate in a context of intense pressure from stakeholders, pharmaceutical industry and consumer organisations to provide funding for new medicines.

In this context, several countries have implemented novel subsidization schemes for new medicines, with the aim of allowing some form of access which is closely managed through agreements set up with pharmaceutical companies. These managed entry agreements have been defined as "*an arrangement between a manufacturer and payer/provider that enables coverage or reimbursement of a health technology subject to specific conditions. These arrangements can use a variety of mechanisms to address uncertainty about the performance of technologies or to manage the adoption of technologies in order to maximise their effective use or limit their budget impact*" [7].

### 1.1. Australia's public funding system for pharmaceuticals

In Australia, a national public pharmaceutical insurance system, the Pharmaceutical Benefits Scheme (PBS), was established in 1953. By 2011, the PBS subsidised around 4000 products. In 2011–2012, PBS government expenditure represented AUD $9193.7 million and 83.4% of the total cost of PBS prescriptions, the remainder being patient contributions [8]. The decision to list new medicines on the PBS is taken by the Federal Minister for Health on recommendation from the Pharmaceutical Benefits Advisory Committee (PBAC), an independent expert body that determines the effectiveness and cost-effectiveness of new medicines. Australia was the first country to introduce an explicit requirement for economic evaluation in its subsidisation assessment process in 1993. Although there is no capped budget for the PBS, any medicine the PBAC recommends for listing that is expected to cost more than $20 million per year in any of the first four years must be approved by Cabinet [9]. Medicines listed in the Schedule can fall into three broad categories: 'unrestricted benefits' for medicines with no restrictions on therapeutic use, 'restricted benefits' for medicines that can only be prescribed for specific therapeutic uses, and 'authority required benefits' for medicines that require prior approval from the Department of Health. Prescribing restrictions may limit use to indications for which the medicine has been deemed effective or cost-effective and may include rules for initiation or continuation of treatment.

In Australia, managed entry agreements have been operating for a number of years. In May 2010, the Australian Government and Medicines Australia, the peak pharmaceutical industry organisation, signed a Memorandum of Understanding which introduced a new type of managed entry agreement [10]. This memorandum focused on the expansion of the price reform policies that had started in Australia in 2007 and included provisions on a 'Managed Entry Scheme' for pharmaceuticals. Clauses 26 and 27 of the memorandum state that the PBAC '*may recommend PBS coverage at a price justified by the existing evidence, pending submission of more conclusive evidence of cost-effectiveness to support listing of the drug at a higher price*" [10]. To support the process, the Australian Department of Health published a framework for the introduction of this form of managed entry agreement [11]. The framework states that a submission would be considered for a Managed Entry Scheme when there is '*a high clinical need for the proposed drug in the indication requested by the sponsor*', and that '*new clinical data would resolve the issues of uncertainty in relation to the extent or value of the clinical effect which would have otherwise prevented an initial positive recommendation*'. This includes the possibility of a randomised controlled trial (RCT)-based managed entry scheme with a trial protocol available at the time of the original submission. The framework also notes other non-RCT level evidence "*may be appropriate, such as data collection for the purpose of confirming cost-offsets in economic analyses*".

In this paper, we examine Australia's past and more recent experience with managed entry agreements for pharmaceuticals.

## 2. Methods

Many terms have been used to describe the various types of managed entry agreements but a common feature of the taxonomies that have been proposed is the distinction between outcome based and non-outcome based agreements [12–14]. In this paper we have used a taxonomy adapted from those developed by Carlson et al. [12] and Ferrario and Canovos [14] that provides a simple classification suitable for the Australian setting. It distinguishes non-outcome based agreements and outcome-based agreements (Fig. 1). Non-outcome based agreements are usually financial in nature and aim to contain the costs without taking into consideration health outcomes. They may include price-volume agreements, discounts, price-capping schemes or dose-capping schemes. Outcome-based agreements have been defined as "*schemes between healthcare payers and medical product manufacturers in which the price, level, or nature of reimbursement are tied to future measures of clinical or intermediate endpoints ultimately related to patient quality or quantity of life*" [12]. The outcome-based agreements may be considered at the patient level and may include outcome-guarantee schemes (e.g. rebates or reimbursement if the medicine fails to achieve the expected results), or conditional continuation schemes. At the population level, coverage with evidence development (CED) schemes have been defined as '*any policy mechanism that links financial support for medical technologies or treatments to a requirement for systematic data collection and analysis with the intent of using that data to modify health policy or clinical decision-making*' [15].

We collected data on non-outcome and outcome agreements operating in Australia from documents publicly available on the Australian government's website including the Schedule of Pharmaceutical Benefits (edition February 2013), which provides details of medicines subsidised by the Australian Government [16] or the Therapeutic Relativity Sheets (edition October 2012) released by the