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Governance of conditional reimbursement practices in the Netherlands

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ABSTRACT

When entering the market, orphan drugs are associated with substantial prices and a high degree of uncertainty regarding safety and effectiveness. This makes decision making about the reimbursement of these drugs a complex exercise. To advance on this, the Dutch government introduced a conditional reimbursement trajectory that requires a re-evaluation after four years. This article focuses on the origins, governance and outcomes of such a conditional reimbursement trajectory for orphan drugs. We find that the conditional reimbursement scheme is the result of years of discussion and returning public pressure about unequal access to expensive drugs. During the implementation of the scheme the actors involved went through a learning process about the regulation. Our analysis shows that previous collaborations or already existing organisational structures led to faster production of the required data on cost-effectiveness. However, cost-effectiveness evidence resulting from additional research seems to weigh less than political, judicial and ethical considerations in decision making on reimbursement of orphan drugs in the Netherlands.

1. Introduction

Orphan drugs target rare diseases, i.e. life-threatening or chronically debilitating illnesses with a low prevalence. Despite the small and often heterogeneous populations associated with these drugs, the number of orphan drugs entering the market has risen in recent years [1,2], amongst others because of supportive EU and US policies. Small patient pools mean that R&D costs need to be recouped from smaller sales, resulting in relative high prices [3]. Moreover, small and heterogeneous patient pools often indicate limited and weak clinical and economic data at time of product launch [4]. Such combination of substantial prices and high degrees of uncertainty leads to complex decision making about reimbursement of orphan drugs. Decisions on reimbursement of these drugs are taken against the background of two opposing perspectives: solidarity [5] versus efficient deployment of limited resources (e.g. [6]).

To deal with this complex decision making at time of market launch, the Dutch government introduced a conditional reimbursement trajectory that requires a re-evaluation after four years. This article concentrates on the *origins*, *governance* and *outcomes* of such a conditional reimbursement trajectory for orphan drugs. Although a wide range of scholars discussed the reimbursement of expensive orphan drugs [3,4,6], this focus is interesting since little has been written about novel reimbursement

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routes. In addition, the societal relevance is reflected by the recurrent debates on innovation capacity of pharmaceutical companies [7] and on consequences for public health budgets and access to medicines [4]. More in general, reflecting on conditional reimbursement is in line with public policy interests to address uncertainties inherent to reimbursement decisions, e.g. in the form of managed entry agreements and risk-sharing schemes [8].

2. Origins of conditional reimbursement

Innovative orphan drugs, such as enzyme replacement therapies, answer to unmet medical needs associated with rare diseases. To benefit from centralising expertise, treatment with these specialised drugs is often initiated in university hospitals. However, centralisation led to concentration of costs in those hospitals, putting pressure on hospital budgets and to misalignment between priorities of hospital management and medical staff. In the end, inequalities in care provision between hospitals, labelled as 'healthcare postcode lottery', could ensue [9].

Problems with expensive specialist drugs entered the Dutch political arena for the first time in 1993 with the introduction of paclitaxel (which is not an orphan drug) and attracted attention of politics and media for over two decades. From 1995 onwards, patient organisations reported inequalities in prescriptions across hospitals and lobbied Parliament to address this issue. This led the ministry to support changes in medical guidelines, subsidies for paclitaxel prescriptions, and a registry to investigate the extent of the problem.

Only from 2001 onwards European-wide registered orphan drugs entered the Dutch market with the introduction of agalsidase alfa and alglucosidase beta for Fabry disease. Because of lacking data on cost-effectiveness and high unmet medical need, the ministry introduced a temporary, dedicated subsidy scheme. After another major political discussion, following problems with reimbursement of trastuzumab, an expensive (non-orphan) breast cancer drug, the minister introduced an instrument on financing expensive medicines that also explicitly included orphan drugs.

Through this policy rule, hospitals obtained 100% compensation for their orphan drug costs and 80% for other expensive drugs. The policy rule applied to those drugs that were included on a positive list. Admission to the list was subject to criteria, including expected cost-effectiveness and budget impact. Due to suboptimal data on cost-effectiveness at the moment of reimbursement decision making [10], inclusion was regarded as temporary, based on incomplete cost-effectiveness and therapeutic value data, and conditional to concrete research plans to enrich and complete the datasets. After four years a re-evaluation would happen, taking into account the produced data on budget impact, therapeutic value and cost-effectiveness in daily practice. Such scheme could be regarded as an example of 'coverage with evidence development' [11].

In August 2012, four years after initiating conditional reimbursement of three orphan drugs for Pompe and Fabry disease (alglucosidase alfa, agalsidase alfa, alglucosidase beta), the first draft re-evaluation reports leaked to the press just before planned release by CVZ. These drafts reported that these drugs were too expensive (with annual treatment costs between 200,000 and 700,000 EUR) relative to the gain in quality of life and life expectancy. This re-evaluation spurred a public outrage in the Netherlands in summer 2012 and reinvigorated public and political debate on (conditional) reimbursement policy as well as a reconsideration of the role of cost-effectiveness in reimbursement decision making.

3. Evaluation of governance and outcomes of conditional reimbursement process

When evaluating the first six years of the conditional reimbursement process (2006–2012; see Appendix A for the methodology of the evaluation), we focused on two perspectives: the efficiency of the re-evaluation processes and the outcomes of these processes.

3.1. Efficiency of governance of re-evaluation process

In the re-evaluation process, the role of CVZ, the ministry and other governmental agencies was confined to defining procedures and assessment, leaving the coordination to a wide range of parties. Formally, the federation of hospitals is responsible for requesting inclusion of a drug on the conditional approval list and for producing additional data. The federation does not have expertise or direct incentives to be prime movers in the process, though. As a result of this clear lack of problem-ownership, in practice two coordination models emerged.

- In the first governance model (applying to orphan drugs labelled '1 in Fig. 1) there is a clear expert centre in one university hospital that treats most patients in the Netherlands and performs research on the disease. The medical specialist-researchers already perform small-scale outcome studies, having good access to the patient population. Frequent and intimate interactions with patient organisations lead to effective research performance, e.g. in terms of patient recruitment and even co-founding of natural history registries.
- The second model articulates pharmaceutical companies as the major players ('2' in Fig. 1). In most cases an acknowledged single expert centre is absent. The companies coordinate and subcontract the required studies in academic hospitals.

In two orphan drug cases neither model applied ('3' in Fig. 1). The interactions between medical specialist-researcher, patient organisation and company were suboptimal or even lacking, and no actor was able to take the lead.

Interview results favour the first governance model in terms of efficiency of conducting additional studies and re-evaluation, and respondents widely agree on the ineffectiveness of the third model. Fig. 1 seems to support this. In this first period after the introduction of the policy rule, the decision-making process on the preparation of the cost-effectiveness studies and assessments by CVZ (black bars) of drugs associated with the medical-dominated