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Do the Current Performance-Based Schemes in Italy Really Work? "Success Fee": A Novel Measure for Cost-Containment of Drug Expenditure

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ABSTRACT

Background: Drug costs have risen rapidly in the last decade, driving third-party payers to adopt performance-based agreements that provide either a discount before payment or an ex post reimbursement on the basis of treatments' effectiveness and/or safety issues. **Objectives:** This article analyses the strategies currently approved in Italy and proposes a novel model called "success fee" to improve payment-by-result schemes and to guarantee patients rapid access to novel therapies. **Methods:** A review of the existing risk-sharing schemes in Italy has been performed, and data provided by the Italian National report (2012) on drug use have been analyzed to assess the impact on drug expenditure deriving from the application of "traditional" performance-based strategies since their introduction in 2006. **Results:** Such schemes have poorly contributed to the fulfillment of the purpose in Italy, producing a trifling refund, compared with relevant drugs costs for the National Health System : €121 million out of a total of €3696 million paid. The novel risk-sharing agreement called "success fee" has been adopted for a new high-cost therapy approved for idiopathic pulmonary fibrosis, pirfenidone, and consists of an ex post payment made by the National Health System to the manufacturer for those patients who received a real benefit from treatment. **Conclusions:** "Success fee" represents an effective strategy to promote value-based pricing, making available to patients a rapid access to innovative and expensive therapies, with an affordable impact on drug expenditure and, simultaneously, ensuring third-party payers to share with manufacturers the risk deriving from uncertain safety and effectiveness.

Keywords: cost-containment, performance-based, reimbursement, risk-sharing.

Introduction

During the last decade pharmaceutical expenditure has rapidly increased, and burdens more than other health care costs in many European countries and in the United States [1]. Oncological care is one of the fields in which spending increased faster, growing up to 21% per annum in recent years [2], because of the introduction of novel high-cost therapies, together with the increase in the prevalence of cancer [3].

Many treatments introduced in clinical practice are molecularly targeted agents [4], whose costs vary between an average of approximately $5,000 (€3,700) to more than $10,000 (€7,400) per month [5], most often exceeding $25,000 (€18,500) per year. These treatments, however, often result in benefits measured in months of survival [6]. In a recent analysis published in *Blood* [7], a large group of experts in chronic myelogenous leukemia pointed out examples of dramatically high costs for antineoplastic drugs such as bosutinib, ponatinib, and omacetaxine, concluding that for many clinical conditions, drug prices do not reflect objective benefits in terms of survival prolongation, degree of tumor shrinkage, or improved quality of life because drug prices for new medicines are mostly set on the basis of price of the most recent similar compound commercially available.

High costs, questionable efficacy, and long-term results of new medicines raised questions about their affordability, application in clinics, and cost-effectiveness [8], leading to the need of adopting cost-containment measures, aimed at reducing expenditure for public health. In Europe, third-party payers have introduced different cost-containment strategies to overcome the problem of public health expenditure, leading to reimbursement agreements in which the burden is shared with pharmaceutical companies and the third-party payer. In an official

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report, the "Good Practices for Performance-Based Risk-Sharing Arrangements (PBRSAs)" Task Force of the International Society for Pharmacoeconomics and Outcomes Research defined such agreements as schemes that "involve a plan by which the performance of the product is tracked in a defined patient population over a specified period of time and the amount or level of reimbursement is based on the health and cost outcomes achieved" [9]. In other words, in a PBRSA, the final remuneration or reimbursement of a pharmaceutical is linked to a previously agreed objective, based on effectiveness or budget impact [10].

The aim of this study was to overview the current PBRSAs approved in Italy so far, where such schemes exist since 2006, and to critically evaluate the impact of their application on drug expenditure. The study also proposes a novel tool for the improvement of cost-containment strategies, called "success fee," an evolution of the performance-based reimbursement concept, already adopted in Italy for the drug pirfenidone, approved for the treatment of idiopathic pulmonary fibrosis.

Reimbursement Schemes in Europe

Although in Europe several reimbursement schemes have been adopted and differently recognized, they can be classified into two broad categories: financial-based schemes and performance/outcome-based schemes [1]. The former category includes "price per volume" (focused on controlling financial expenditure, with pharmaceutical companies refunding overbudget situations) and "patient access scheme" (including free drugs or discounts for an agreed period to enhance the value of new medicines and improve the possibility of their funding/reimbursement). PBRSAs are established "between a payer and a manufacturer of pharmaceuticals, devices or diagnostics, where the price level and/or the revenue is related to the future performance of the product in either a research or a real-world environment" [11].

PBRSAs link the reimbursement or price of the new technology/medication to the health outcomes derived from its utilization in the "real world": reimbursement thus depends on future assessment of clinical end points [12].

Within the European Union, several countries are currently using some form of PBRSAs, most of them financially-based, because the performance-based schemes adopted so far have shown critical difficulties in terms of applicability [10]. United Kingdom, The Netherlands, France, and Italy reported a larger use of PBRSAs than did other countries within the European Union [9].

Reimbursement Schemes in Italy

The Italian National Health System (NHS) has adopted several instruments to manage budget impact, uncertain clinical outcome, and appropriate use of medicines. These instruments include discounts (possibly hidden discounts), price-volume agreements, performance-based schemes, therapeutic plans, "AIFA notes," that is, restriction of prescribing centers, and monitoring registries used to collect data about drug safety and effectiveness [9]. The AIFA notes limit reimbursement of the relevant drugs to population subgroups. The monitoring registries have represented, since 2005, an advanced tool to ensure not only prescription appropriateness but also the applicability of PBRSAs [13]. Most of the drugs included in the registries were approved under a centralized marketing authorization (often rapid and/or conditional approval) and are specifically biologics and/or high-cost drugs. Reimbursement strategies are made to ensure not only a rapid patient's access to drugs but also cost control. In fact, the adoption of a PBRSA is commonly associated with a faster patient's access [14]. When price and reimbursement are negotiated by AIFA and the relevant company, the choice of the type of PBRSA to be adopted depends on the data available on the efficacy and safety of drugs, as well as on pharmaceutical products' characteristics and on the availability of alternative therapies [10].

Italy has its own classification system for PBRSAs, which includes the following three categories:

- "cost sharing," which is a discount for initial cycles of treatment for all eligible patients;
- "risk sharing," which sets a partial reimbursement for eligible nonresponders only, after a clinical evaluation; and
- "payment by results," which sets a total reimbursement by the manufacturer for nonresponders.

The system of applying an initial discount to all eligible patients used in the "cost-sharing" scheme is simpler to manage than the system of reimbursement for nonresponders used in the "risk-sharing" and "payment-by-results" schemes, and it is applied when reliable data on the efficacy and safety of the medicine are available. Usually, risk-sharing and payment-by-results schemes are applied in the case of medicinal products whose risk-benefit ratio has a greater degree of uncertainty, thus requiring a definition of nonresponders that derives from the characteristics and the results of pivotal clinical trials [13]. For each eligible patient, a file is opened in the registry and followed up until reevaluation. To be considered eligible for reimbursement, it is critical that every patient's file is full and closed at the end of treatment. The distinction between responders and nonresponders is based on the outcome recorded in the patient's file, according to the respective negotiation agreement.

Table 1 lists the drugs subjected to PBRSAs in Italy at the date of December 31, 2012. Most of these drugs have been approved for oncological care.

Analysis of Data Available in Italy

We based our analysis on the data published in the annual report "Drug Use in Italy: National Report 2012" by Osservatorio Nazionale sull'impiego dei Medicinali [13]. Table 2 describes the total amount of money that has been reimbursed by the companies, as of 2012, for the 22 drugs for which risk-sharing schemes have been activated since their establishment in 2006. Despite the application of the three schemes adopted in Italy, it appears that the amount of money refunded through the reimbursement procedures is trifling: €121 million out of a total of €3696 million (i.e., 3.3%) [15].

Focusing on expenditure/reimbursement data relative to the market of drugs under PBRSAs for the year 2012, we see that €823 million has been paid by the NHS for the treatment of patients. Out of this amount, only €46.3 million (5.6%) underwent the reimbursement procedures, which means that 94.4% of the expenditure was not considered for refund. Reasons accounting for such a high percentage of unrequested reimbursements may be found, at least in part, not only in the high percentage of patients still under treatment and in interruptions of treatment for reasons other than the ones provided in the negotiation agreement but also in patients' files that have not been closed because of the health care center inefficiencies, thus preventing the activation of the reimbursement procedure. Moreover, out of €46.3 million expected to be refunded after the reimbursement procedure activation, only €31.3 million (67.7%) has actually been refunded by the companies (Fig. 1) [13], while the remaining €15 million (32.4%) was not reimbursed because of lack of refund request by hospitals, inefficiency of administrative centers or management/treatment errors (5 million; 10.8%), and rejection of refund requests by the companies (10 million; 21.6%) likely because of other unspecified formal issues.

Excluding the amount eligible for refund of two of the drugs included in the PBRSAs (sorafenib and temsirolimus) that are subject to a mixed mechanism of reimbursement (based on