EVALUATING NATIONAL HEALTHCARE SERVICE SPENDING WITH PFS-BASED MODELS VERSUS TRADITIONAL REIMBURSEMENT SCHEMES FOR BREAST AND LUNG CANCER IN ITALY

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Introduction

The introduction of novel and often expensive therapies poses important challenges for payers, especially when limited evidence of the added therapeutic value exists at the time of pricing and reimbursement decisions.[1] In alternative to traditional reimbursement schemes based on confidential discounts over list prices, outcomes-based managed entry agreements (OBMEAs) can be negotiated between pharmaceutical manufacturers and payers to manage the clinical and financial uncertainty related to the impact of a drug.[1-3]

In Italy, the most widely adopted OBMEA is the Payment-by-Result (PbR) scheme,[4] for which the pharmaceutical manufacturer fully refunds the National Healthcare Service (NHS) when the patient does not achieve a specified treatment response, such as progression-free status, within a pre-defined time period.[1]

Despite their relevance, the body of current evidence on the financial outcome of these type of agreements is sparse,[1] and their eventual economic convenience for the NHS is poorly investigated.[5] This study aims to contribute to the debate by assessing the scenarios making progression-free survival (PFS)-based PbR schemes a convenient alternative to traditional reimbursement models for drugs indicated in the treatment of breast and lung cancer in Italy.

Methods

Phase 3 clinical trials having PFS as primary endpoint and reporting mature Kaplan-Meier (KM) data for drugs reimbursed in Italy in the last three years (2021-2023) as first-line (1L) treatments for the deadliest solid tumors[6] for women (breast cancer) and men (lung cancer) were identified through an IQVIA proprietary database on Italian negotiation dynamics.

For each drug, a PFS-based PbR scheme with a duration of 12 months was modeled for the purpose of the present analysis. Cost per patient assuming the median treatment duration reported in the corresponding trial was computed both under the modelled scheme and under a traditional reimbursement scheme, in two steps (*Figure 1*):

1. The PFS curve reported in the trial was digitized with the publicly available WebPlotDigitizer online software to retrieve the PFS value at 12-month and assess probability of reimbursement *p* with the modelled scheme;
2. The cost sustained by the Italian NHS under the two schemes was estimated as follows:
   - For the first year of treatment (or for a period of time equal to median treatment duration, in case of durations below 12 months) full reimbursement was assumed with probability 100% under the traditional reimbursement scheme and with probability *p = 12-month PFS value* retrieved in step 1 under the PbR scheme;
   - For the remainder duration of treatment (only in case of drugs with median treatment durations longer than 12 months) full reimbursement was assumed with probability *p = 12-month PFS value* under both schemes, since patients experiencing disease progression within 12 months are assumed to discontinue treatment, and therefore the cost for the remaining treatment duration is not borne by the NHS, regardless of the reimbursement scheme adopted.

Lastly, for each trial, a confidential discount was assumed to be applied to the cost estimated under the traditional scheme. By exploring all possible discount values between 0.0% and 100.0%, scenarios making the 12-month PFS-based PbR scheme an economically convenient option were assessed.

Results

Five clinical trials were included in the study, three on lung cancer and two on breast cancer treatments.

Except for one case, all of them reported a median treatment duration longer than one year and presented a 12-month PFS value higher or equal than 0.6, as shown in *Table 1*. Estimations of cost per patient associated with each trial's median treatment duration under the OBMEA scheme ranged from € 85.6k to € 23.1k, respectively for longest and shortest median treatment duration (*Figure 2*).

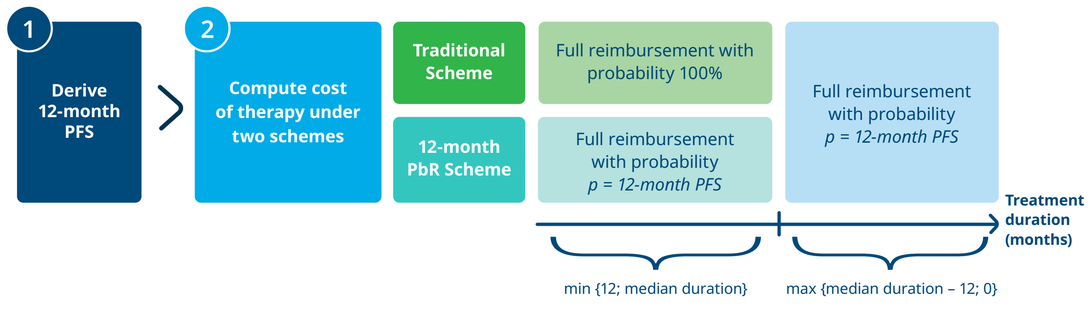
As represented in *Figure 3*, the 12-month PFS-based reimbursement scheme always resulted cost-saving with respect to the traditional one when considering discount values applied over list prices below or equal to 18.0%, while it emerged as a cost-saving alternative for at least 2 drugs out of 5 when increasing the discount up to 34.7%. Even compared to confidential discounts greater than 34.7% and lower or equal to 61.7%, the OBMEA scheme remained a convenient option for one drug. For this drug, the trial reports both the lowest median treatment duration and the lowest 12-month PFS value, evidencing the scheme's high potentiality in mitigating the financial impact of poor drugs' performance. Only when increasing discount values above 61.7%, the traditional reimbursement scheme consistently appeared as the most convenient alternative for all the five cases considered in the present analysis.

Conclusions

The analysis showed that, when introducing 1L treatments for either breast or lung cancer, 12-month PFS-based PbR reimbursement schemes may mitigate the financial impact on the Italian NHS spending compared to traditional schemes, while linking the reimbursement to drugs' value. In particular, the potential financial convenience brought by this type of agreements is more pronounced in case of drugs reporting poorer performance (i.e., lower median duration and PFS value).

More comprehensive analyses incorporating real-world data on drugs' performances and replicating the approach with drugs for different indications may allow to extend generalizability of results.

Figure 1. ***Graphical representation of the adopted methodology***



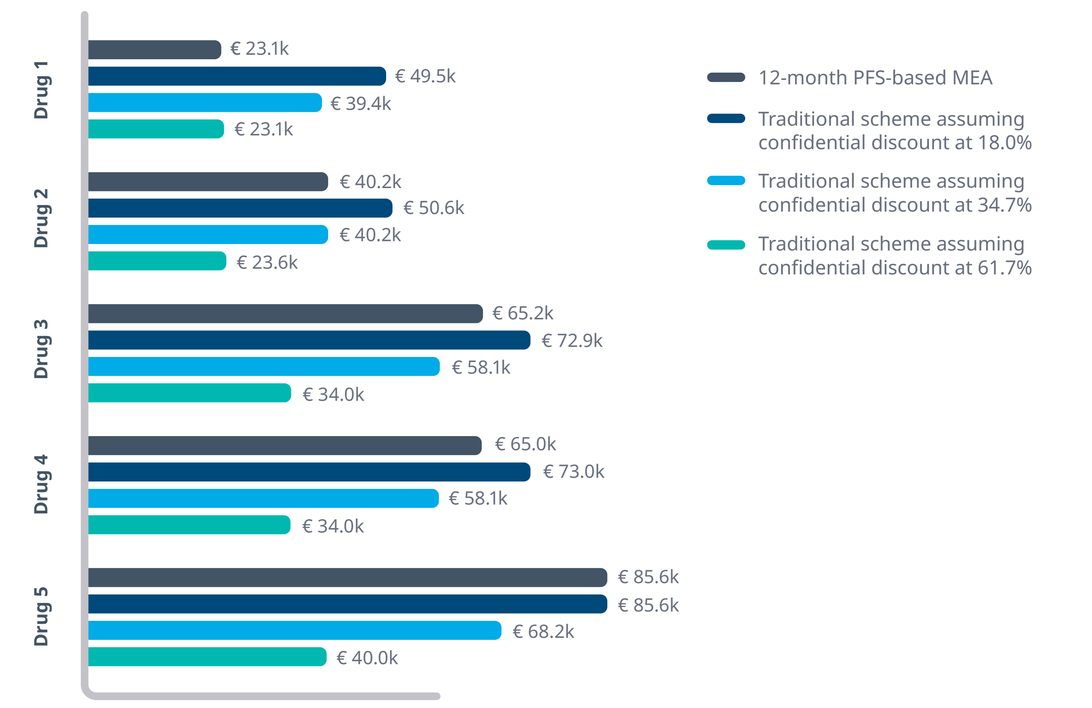
Acronyms: PFS = Progression-Free Survival; PbR = Payment by Results

Table 1. ***Drugs and relative parameters considered for the present analysis***

	THERAPEUTIC AREA	MEDIAN TREATMENT DURATION	12-MONTH PFS VALUE
Drug 1	Breast cancer	~ 6 months	~ 0.4
Drug 2	Lung cancer	~ 15 months	~ 0.6
Drug 3	Lung cancer	~ 16 months	~ 0.7
Drug 4	Breast cancer	~ 18 months	~ 0.6
Drug 5	Lung cancer	~ 24 months	~ 0.7

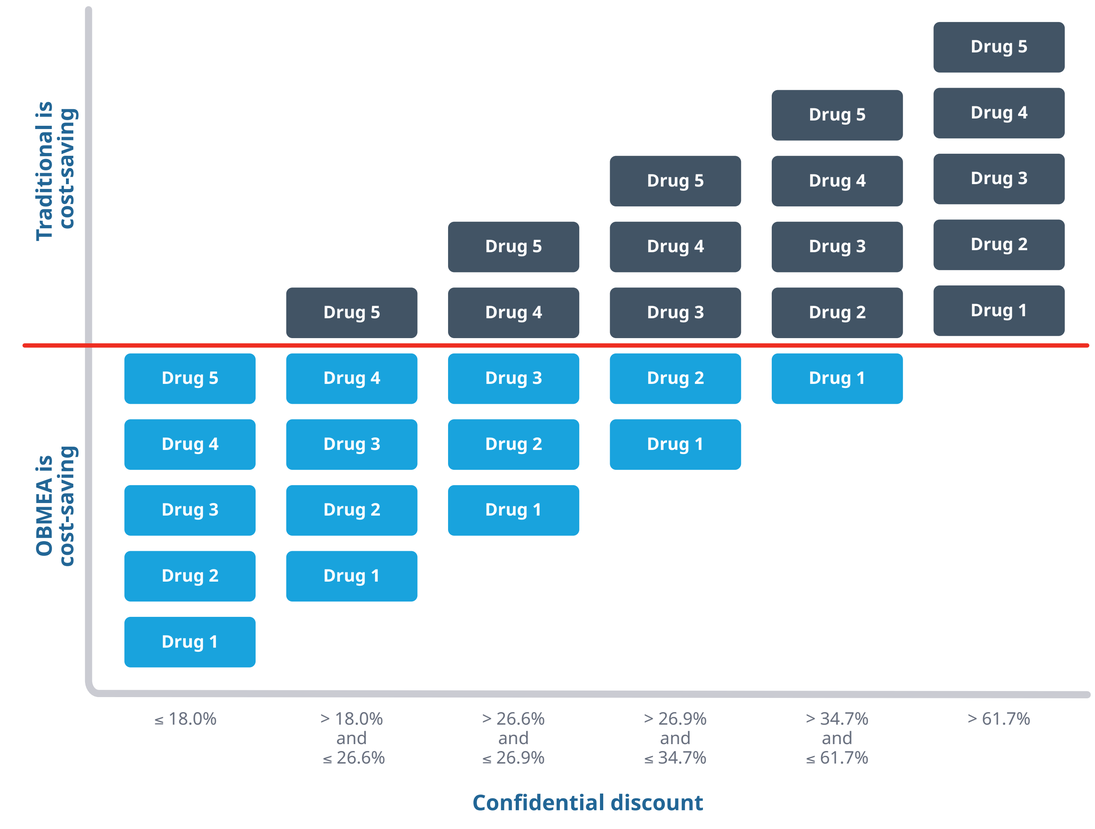
Acronyms: PFS = Progression-Free Survival

Figure 2. ***Estimated cost for the Italian NHS considering different reimbursement schemes***



Acronyms: NHS = National Healthcare Service; MEA = Managed Entry Agreement; PFS = Progression-Free Survival

Figure 3. ***Comparison between traditional and 12-month PFS-based reimbursement schemes***



Acronyms: OBMEA = Outcome-Based Managed Entry Agreement; PFS = Progression-Free Survival

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