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Do Patient-Reported Outcomes Influence Drug Prices? Evidence from the Italian Pricing and Reimbursement System

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INTRODUCTION

- Patient-reported outcome measures (PROMs) are increasingly used in clinical trials and regulatory processes to capture patients' perspectives on symptoms and Health-Related Quality of Life.
- However, while the relationship between drug prices and "hard" clinical endpoints, such as overall survival has been explored, the role of PROMs in pricing and reimbursement decisions remains underexplored.
- The objective of this study is to investigate whether the presence and characteristics of PROMs in regulatory submissions are associated with drugs' negotiated prices, specifically considering first submissions corresponding to the initial national assessment and evaluation of each drug.
- The focus of the current study is Italy, which is used as a case study, although Germany will also be considered as a second country as part of the larger study.

METHODS

- **Sample of drugs**: All drugs authorized by the European Medicines Agency (EMA) between 2017 and 2023 were initially considered.
- **Data sources & items**: European Public Assessment Reports (EPARs), for data on clinical and regulatory features, such as drugs' characteristics, ATC, orphan designation, and presence, hierarchy (primary vs. secondary), and type (generic vs. specific) of PROMs; Farmadati for ex-factory prices.
- **Approaches for measuring drug utilization**: 1) price per Defined Daily Dose (DDD; N=279), for drugs with DDD assignment; estimated cost per full treatment cycle/year (for drugs lacking a DDD; N=114), whit treatment durations being based on posology indications, body weight assumptions, and median progression-free survival.
- **Data analysis**: Logarithmic regressions were performed by approaches to drug utilization to examine the association between price and PROMs. Analyses were performed in STATA.

RESULTS

Overview of the sample:

- **393** drugs considered:
  - **21%** orphan designation
  - **16%** generic
  - **10%** biosimilars
- **54%** presence of PROMs:
  - **6%** generic measures
  - **16%** disease-specific measures
  - **30%** both
- PROMs **by type of endpoint**:
  - **5%** primary endpoint
  - **33%** secondary endpoint
  - **8%** exploratory endpoint
- PROMs **by therapeutic class**:
  - **51%** in ATC L (antineoplastic and immunomodulating agents)
  - **10%** in ATC N (nervous system)
  - **9%** in ATC A (alimentary tract and metabolism)

Logarithmic regression – sub-sample of drugs with DDD
(similar insights for sub-sample without DDD)

Robust regression				**Number of obs**	**= 279**	
				F (14, 264)	**= 1.86**	
				Prob > F	**= 0.0304**	
ln_prix	**Coefficient**	**Std. err.**	**t**	**P>t**	**[95% conf.**	**interval]**
PROM presence	1.65870	0.93826	1.77	0.078	-0.18872	3.50612
PROM number	-0.28359	0.19436	-1.46	0.146	-0.66628	0.09909
endpoint_1	-1.27118	0.72264	-1.76	0.080	-2.69404	0.15168
endpoint_2	-0.81568	0.53481	-1.53	0.128	-1.86871	0.23735
endpoint_other	-1.00261	0.71327	-1.41	0.161	-2.40703	0.40181
PROM_generic	0.44740	1.03266	0.43	0.665	-1.58589	2.48069
PROM_specific	-0.64725	0.91860	-0.70	0.482	-2.45596	1.16146
PROM_both	-0.02182	0.89567	-0.02	0.981	-1.78538	1.74174
year						
2018	0.35958	0.39056	0.92	0.358	-0.40943	1.12860
2019	0.77218	0.47792	1.62	0.107	-0.16883	1.71319
2020	0.67552	0.40011	1.69	0.093	-0.11230	1.46333
2021	0.97821	0.42200	2.32	0.021	0.14731	1.80912
2022	0.32027	0.46663	0.69	0.493	-0.59852	1.23905
2023	1.45728	0.66769	2.18	0.030	0.14261	2.77195
_cons	3.19009	0.31515	10.12	0.000	2.56957	3.81061

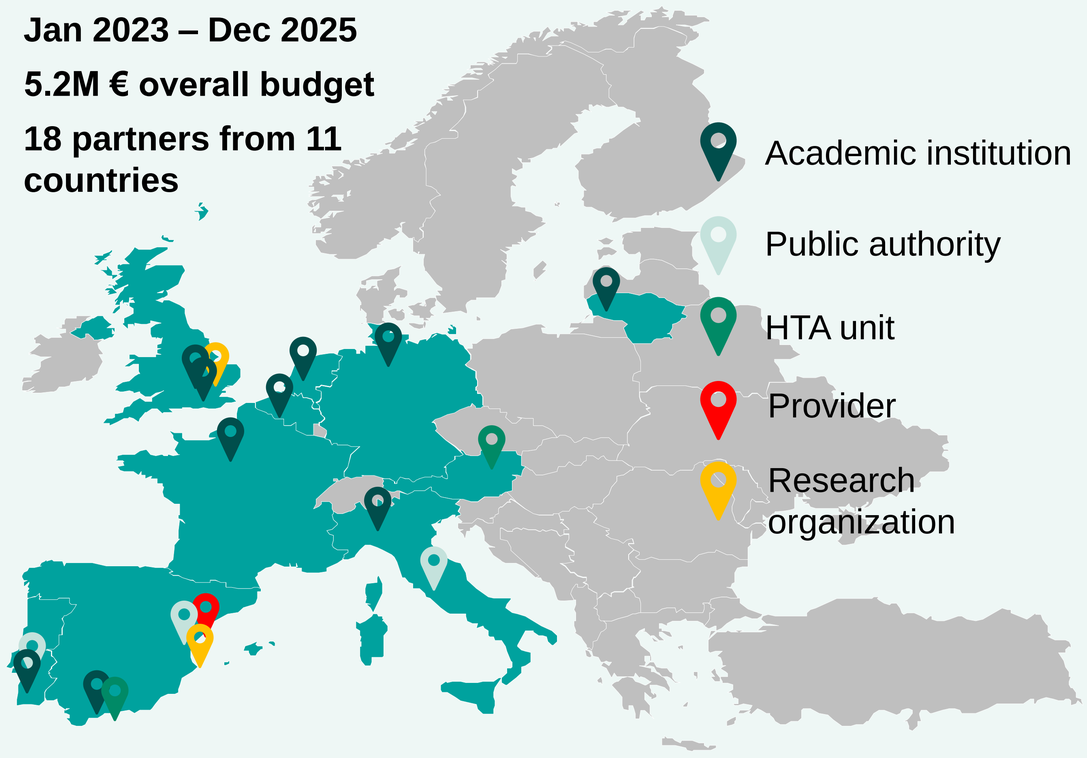
Discussion & policy implications:

- As clinical endpoints show convergence across new therapies (particularly in oncology), PROMs could discriminate products in terms of their added value.
- Our preliminary findings suggest that PROMs do not currently influence pricing negotiations in Italy.
- The lack of impact on pricing may discourage manufacturers from investing in the systematic collection of PROMs, unless clearer incentives mechanisms are established within pricing frameworks.
- Further analyses will compare the impact of both PROMs & «hard enpoints» (e.g., OS) on prices, and will include German prices.

HEALTH INNOVATION NEXT GENERATION PAYMENT & PRICING MODELS **(HI-PRIX):**

Balancing Sustainability of Innovation with Sustainability of Health Care

- **Jan 2023 – Dec 2025**
- **5.2M € overall budget**
- **18 partners from 11 countries**



WP1 Mapping of payment and pricing schemes for health innovation in the EU: implementation, barriers and enablers

WP2 Role of Public Contributions to the Development of Health Innovations and its Integration in Value Assessment and Pricing / Reimbursement Decisions

WP3 Widening the scope of economic evaluations for pricing and reimbursement decisions: the role of indirect medical and environmental costs

WP4 Pricing dynamics throughout the lifecycle of pharmaceutical products

WP5 Novel payment schemes and methods and planning for purchasing and delivering services that incorporate novel technologies or products

WP6 Impact of innovative payment schemes on long-term competition in health technology markets, in particular the pharmaceutical market

WP7 Incentives for pharmaceutical innovation and equitable access to innovation

WP8 Equity-issues mitigation strategies in innovation pricing and payment models

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