Does HTA undermine the goals of EMA authorization pathways? Time to availability of drugs licensed under conditional marketing authorization compared to standard marketing authorization

HPR99

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INTRODUCTION

- To expedite the drug approval process, the European Medicines Agency (EMA) has implemented programs, like the **conditional marketing authorization (CMA)**, that grant access based on incomplete evidence at launch.
- **Incomplete clinical evidence** on a drug's efficacy can be attributable to a variety of factors, such as ongoing pivotal studies at launch, single arm trials, or the use of surrogate endpoints.
- However, while EMA focuses on the **overall risk-benefit ratio** of treatments, national payers in the European Union (EU) focus on their **long-term benefits, cost-effectiveness and budget impact**, thus reflecting a misalignment in incentives and missions.
- This study investigated the **time to availability of drugs** approved with CMA, compared to standard marketing authorization (SMA), **in Italy, Germany, and Spain**.

METHODS

Time to access

"*inclusion of centrally approved medicines on the public reimbursement list in a country*" (EFPIA WAIT indicator)

From EMA approval to assignment of a reimbursement class (A, H, or C). *Source*: Farmadati

From EMA approval to date of «first inclusion in the package». *Source*: Lauer-Taxe

From EMA approval to inclusion in the public reimbursement system. *Source*: Bifimed

Sample of drugs

Conditionally approved drugs (CMA)

Drugs licensed under CMA from 2006 (inception of the program) to 2022.

65 new active substances

Standard approval drugs (SMA)

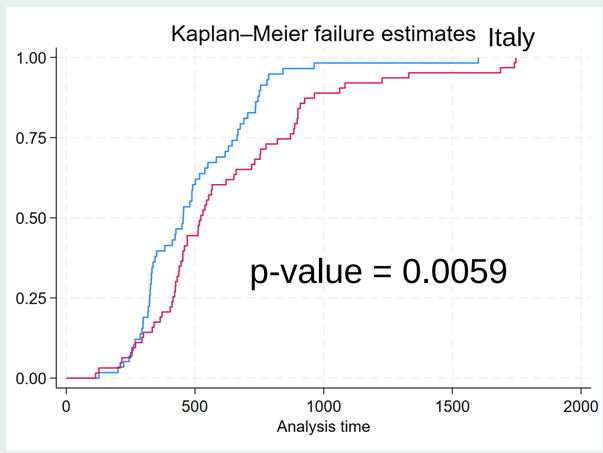
Comparable group of SMA drugs, based on i) ATC codes, and ii) approval dates.

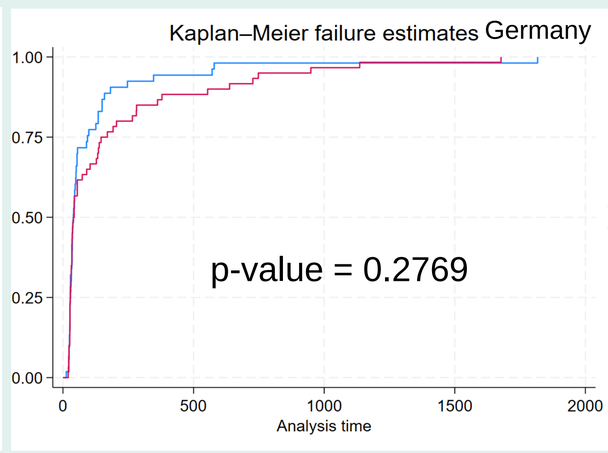
62 matched drugs

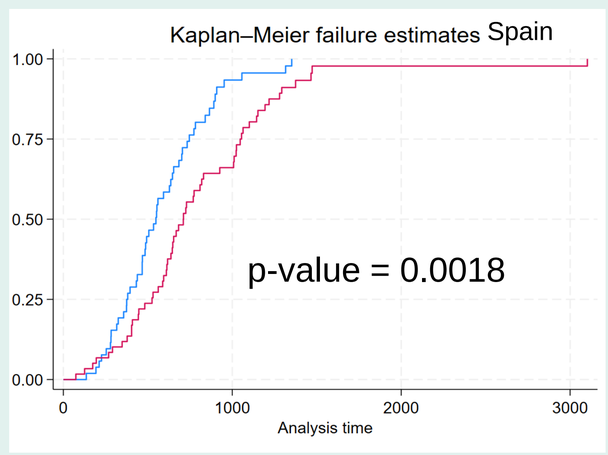
RESULTS

	Italy				Germany				Spain			
	CMA		SMA		CMA		SMA		CMA		SMA	
	N	*Median*	*N*	*Median*	*N*	*Median*	*N*	*Median*	*N*	*Median*	*N*	*Median*
Total	62	520	58	455	59	43	54	39	58	696	52	541
Cancer drug												
Yes	44	470	41	454	43	36	40	37	41	668	39	534
No	18	707	17	488	16	80	14	49	17	774	13	548
Orphan status												
Yes	29	470	21	480	26	38	21	38	26	678,5	20	603
No	33	553	37	454	33	43	33	40	32	696	32	476
ATMP status												
Yes	5	517	3	690	5	91	3	150	6	708	1	506
No	57	523	55	450	54	39	51	37	52	696	51	548

Notes: **N**=number of drugs in each category; **Median**=median number of days from EMA approval to inclusion in public reimbursement list in Italy, Germany, and Spain. Differences in medians were tested with **Kruskal-Wallis Test**





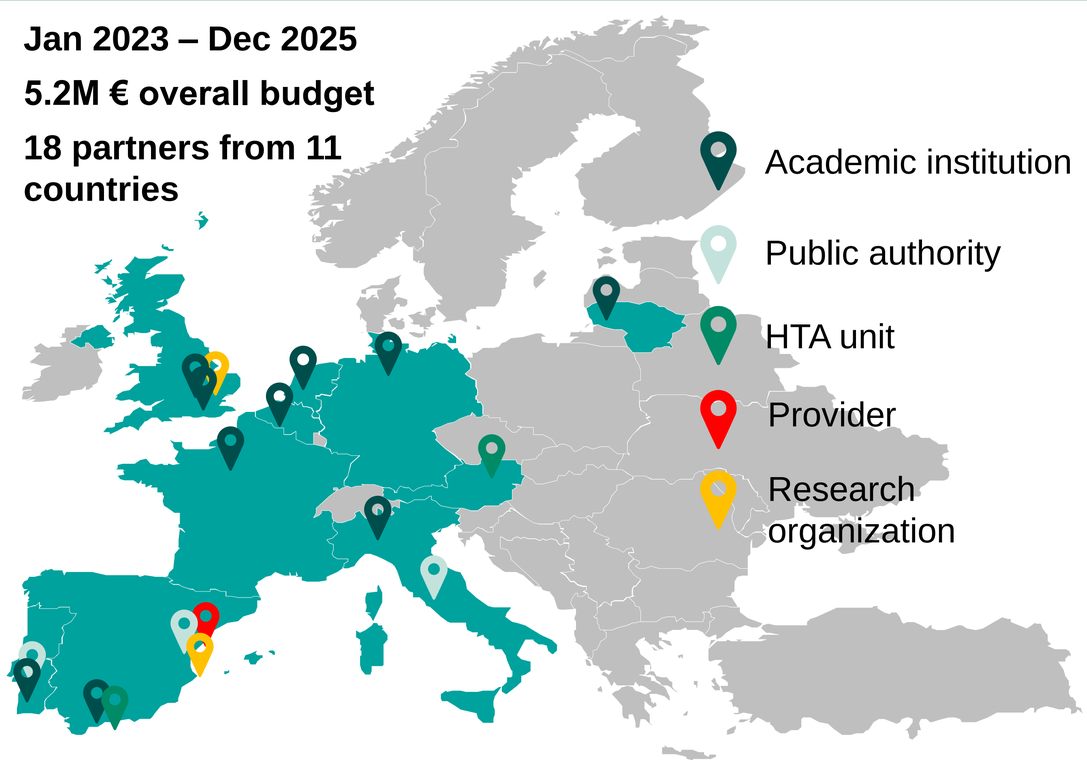


The **EMA approval process is being accelerated**; **payer access is not**. Delays in payer coverage and access in major EU nations are counteracting the intent of specific authorization pathways, like CMA.

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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