

Access through CUPs and NPBs

Opportunities, Barriers & Inequities

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Disclosure



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No conflicts of interest



Background

Prior to EU Marketing Authorization:

- CUP: for groups of patients
- NPB: for individual patients

European rules and regulations:

Varying implementation

Aim study:

Explore differences in BE, FR, GE, NL, NO, CH, UK



Methods



Policy analysis of CUPs and NPBs

Regulatory and reimbursement aspects



**In-depth examination of anticancer
and orphan medicines**
EU authorized in 2021 and 2022

Availability, duration, geographic
distribution



Results: Key features

CUP	NPB
Designed for patient groups	Designed for individual patients: BE, FR, GE, NL, NO, UK • Small quantities: CH • Prescriber: BE • Individual clinics or hospitals: NO
Initiated by manufacturer	Initiated by physician (or pharmacist, or manufacturer (NL))
Nationally regulated	Less regulated: • Centralized (FR, NO, UK, NL) • Decentralized (CH, GE, BE)
Big differences in procedures and duration	Variation more pronounced



Results: Who Pays?

	CUP	NPB
Medicine costs	Cost often covered by manufacturer, but not guaranteed	Unclear/limited information: Costs often fall on hospital or patient
	NL/UK: patient payment unacceptable	GE/CH: no regulation; patients/hospitals often pay
	BE/GE/NO: patient payment forbidden	
	FR/CH: no regulation; patients/hospitals pay	
	FR/BE: reimbursement options	Real financial risks and inequalities
Additional costs (e.g.. hospitalization, pharmacy dispensing fees, outpatient care)	Generally not regulated: BE: Patient GE: Healthcare insurance	Unclear



Results: Access in Practice

- 59 Cancer/orphan medicines EU authorized in 2021 and 2022
 - 22 (37%) had a CUP
 - Most CUPs in 1 or 2 countries
 - Average CUP duration 391 days (variation: 74 days to 981 days)

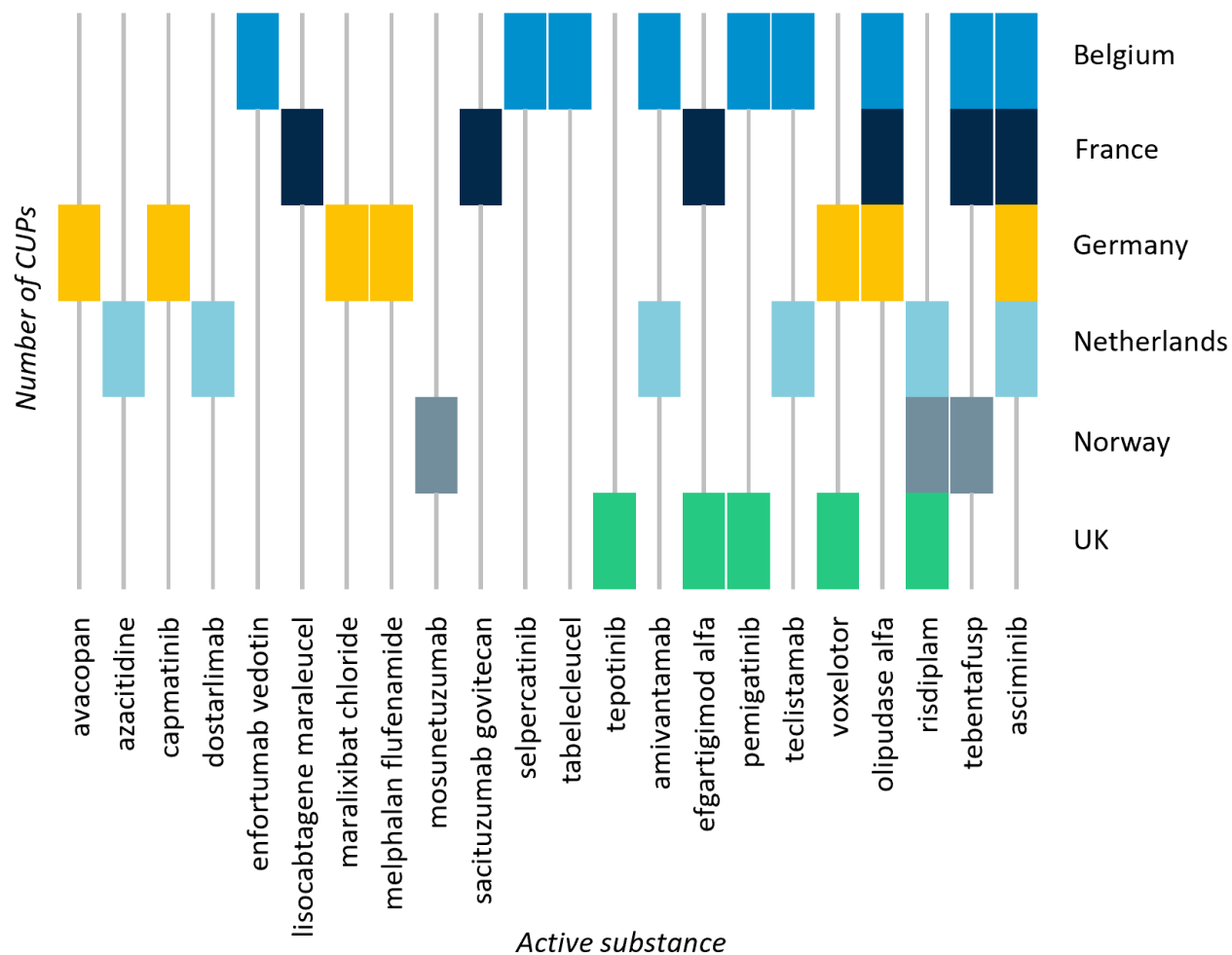


ACCESS DEPENDS HEAVILY ON COUNTRY OF RESIDENCE



In-depth Examination of CUPs

Number of CUPs for anticancer and orphan medicines EU authorized in 2021 and 2022



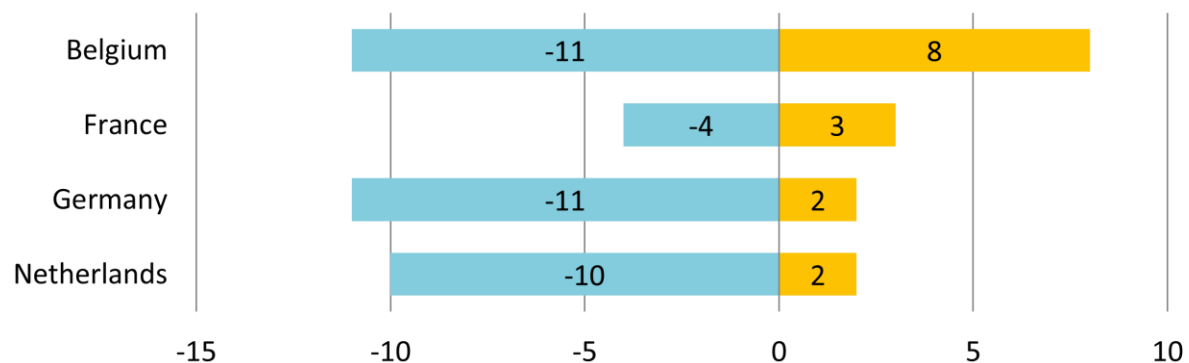
→ Available in 1 to 4 countries



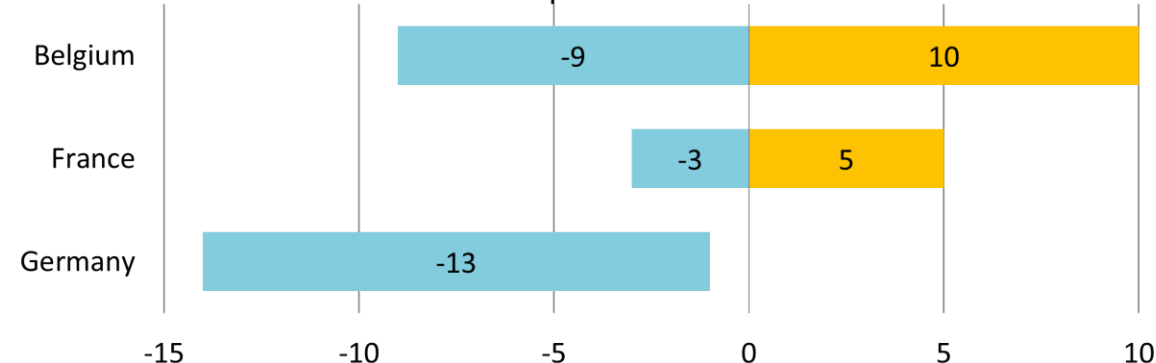
In-depth Examination of CUPs

Duration of CUPs available in at least three countries

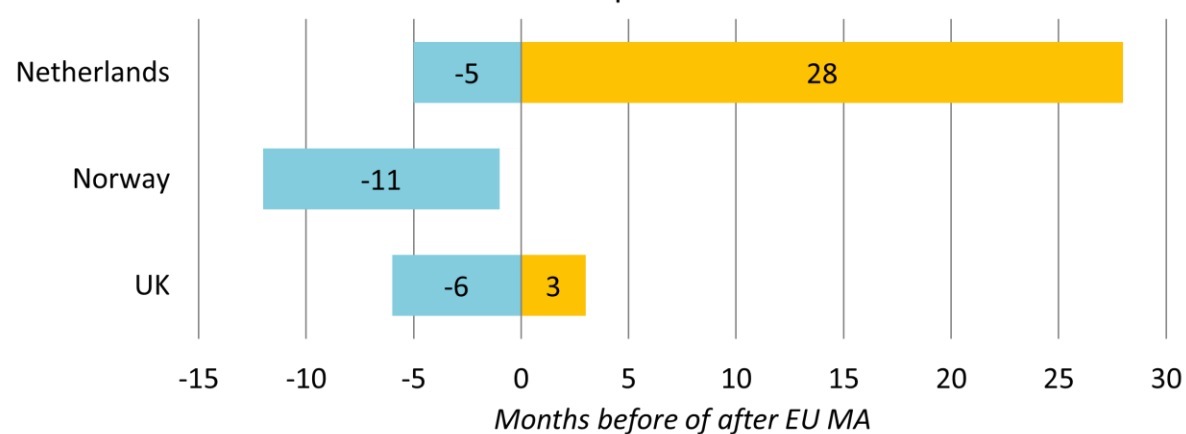
A. asciminib



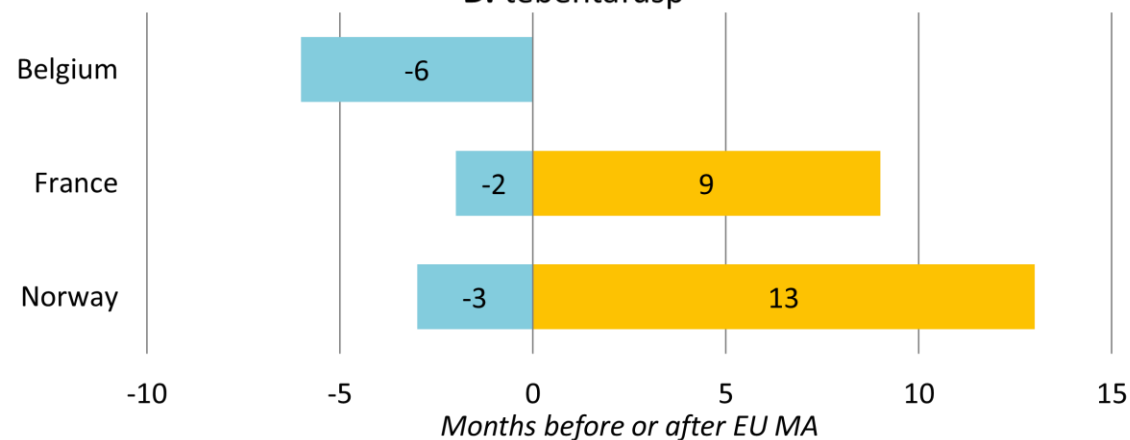
B. olipudase alfa



C. risdiplam



D. tebentafusp





Consequences of Inequities



Patients may:

Travel abroad (if able)
Try to self-fund (ethically problematic)



Physicians face:

Administrative burden
Uncertainty
Reluctance to raise hope



Systemic risk:

Lack of transparency (especially NPBs)
Manufacturers may pull back from CUPs if HTA takes too long



Recommendations:

- Align national systems across Europe
- Increase transparency (especially for NPBs)
- Public registries (with caution to avoid promotional use)
- Prioritize clarity over number of CUPs
- Encourage CUP routes over NPB routes



DEVELOP MANAGED ACCESS PROGRAMMES like ODAP for

→ **Collaboration and real-world data collection**

→ **Support HTA & Pricing Decisions**



Discussion

How do we balance?

- The urgency of early access
- With safety, affordability, and fairness

When evidence and price are still uncertain:

- What level of risk and cost is acceptable?
- How do we ensure equity across all patients, regardless of income or location?

 Call to revision of Pharmaceutical Regulation



Thank you....Coming soon in ESMO-Open

Access to anticancer and orphan medicines through compassionate use programs and named patient basis in seven European countries

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