Current Changes In Europe That Impact Orphan Drugs Access: A Focus On The Latest Reforms In The EU4 and England

HTA140


AUTHORS: Andrea Bernardini, Nadia Al Lahiq, Joanna Fernandes, Olivia Johns, Divya Malia, Akshay Kumar

Objectives

Post-COVID, healthcare systems are balancing the need to improve health outcomes with expenditure.

Healthcare and payer policy reforms have been approved or are being discussed in major European markets: Germany, France, Italy, Spain and England.

This study outlines the evolving access landscape and potential implications for orphan drug (OD) access.

Methods

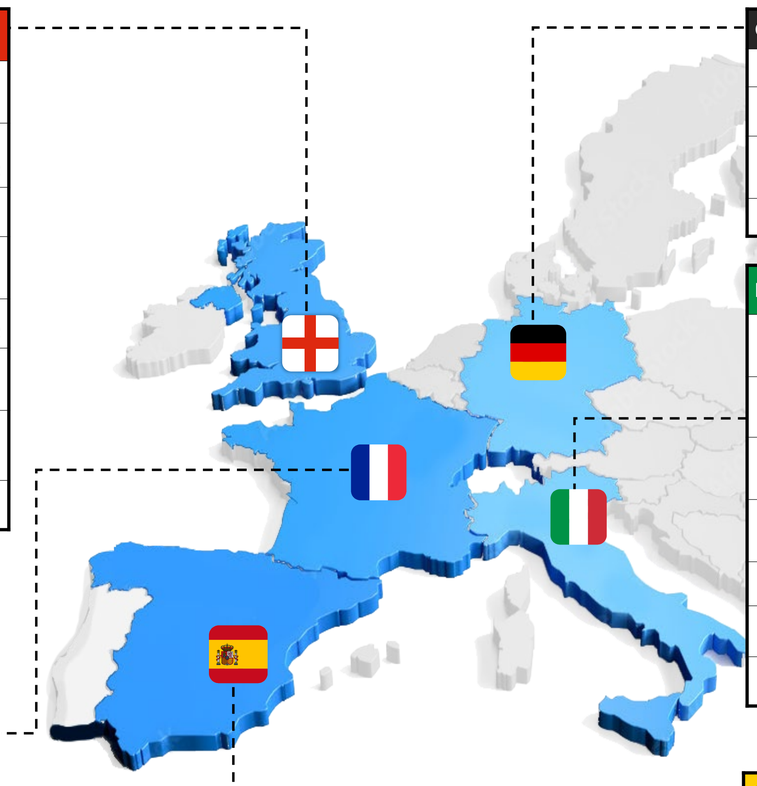
1. For each market, qualitative and quantitative analyses were performed on information collected via targeted literature review of publicly available sources: national authority websites, peer-reviewed articles, and grey literature.
2. Findings were segmented into policy scope, policy occurrence likelihood and OD access impact. Policy scope comprised 4 categories: access, pricing, assessing methodology and wider healthcare policy. Occurrence likelihood was subdivided into occurred, expected, and discussed reforms.
3. The OD access impact was categorised as high, intermediate, and low. A qualitative analyses of the relationship between likelihood of occurrence and impact to OD access pinpointed reforms likely to change the OD landscape.

Results

Reforms that will impact OD access have been identified in the EU4 + England

ACCESS | ASSESSING METHODS | PRICING | WIDER POLICY

England	Detail	Areas
1 IMF	Annual budget of £340mil, aims at temporary reimbursement of non-oncology therapies with clinical uncertainties while additional data is collected	
2 Changes to HST eligibility	Reduction to 4 eligibility criteria (prevalence of disease, number for eligible patients, impact on QoL, lack of treatment options)	
3 MHRA joining IAC	Collaboration with Canadian, Singaporean, Swiss and UK-wide regulatory bodies	
4 HTA access tool ILAP	Tool to aid companies launching drugs that need complex HTA, have commercial challenges and introduce radical changes to established care pathways	
5 Disease severity weighting	A new disease severity modifier will replace the end-of-life modifier in establishing a QALY threshold	
6 NICE RWE framework	Part of NICE Strategy 2021-2026, push for integrating RWE into NICE recommendations; partnership of NICE with RWE international research bodies	
7 Greater acceptance of uncertainty	Case-by-case deliberative decision for committees; it could include use of surrogate endpoints	
8 Proportionate appraisal	New HTA-Lab for innovative drugs meeting significant UM; shorter & less-resource-intensive process piloted for straightforward evaluations	



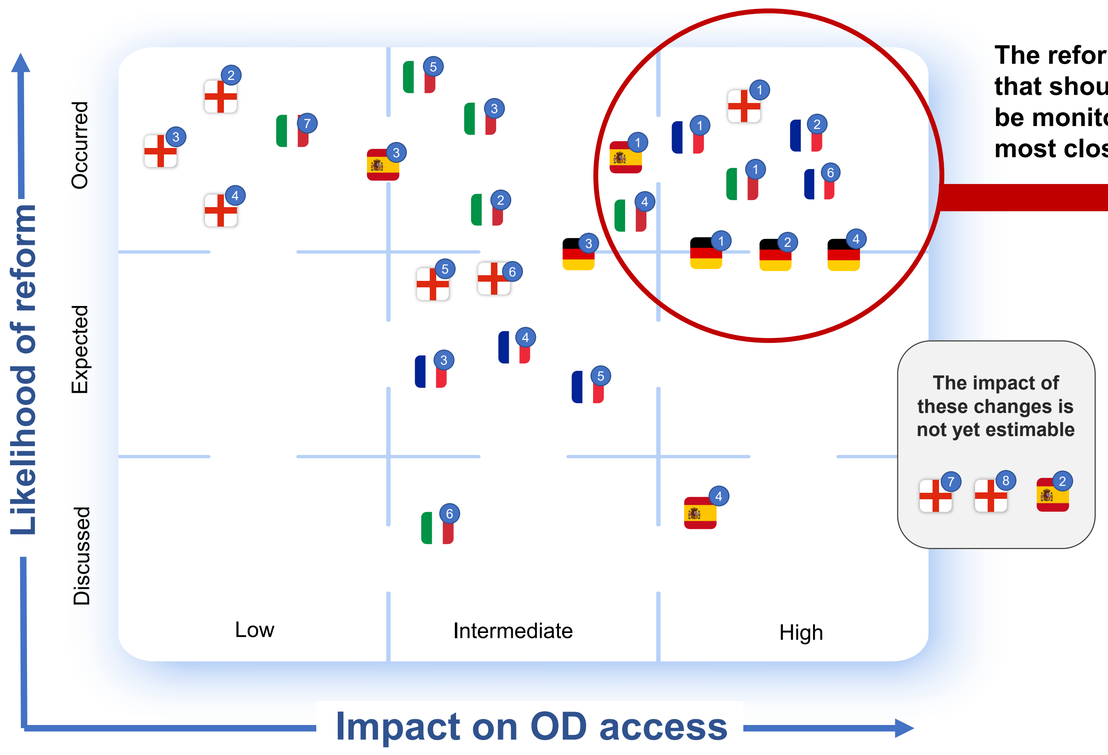
Germany	Detail	Areas
1 Implementation of PVAs or caps		
2 Standard AMNOG evaluation for ODs with annual sales >€20m	Reduction from the current €50m	
3 Additional mandatory ~15% discount for certain combination therapies	The 2nd manufacturer is forced to take a discount even if they don't want to be part of the combo	
4 Reducing the AMNOG free pricing period to 6 months		

Italy	Detail	Areas
1 Updated P&R guidelines	Disclosure of list prices, discounts and MEAs agreed in EU+UK markets; PE analysis required for ODs; simplified PR negotiation for Law 648/96 inclusion	
2 OD regional access	Regions to guarantee OD access to orphan drugs within 2 months (previously 6 months)	
3 Consolidate Law on Rare Diseases	PDTA for rare disease patients to choose appropriate treatment; RD patients solidarity fund of €1mil per year; AIFA 5% fund to increase by +2%	
4 AIFA 5% fund updates	Exclusion of drugs excluded by the CTS from Law 648/96, drugs with repeated use might be temporarily included by AIFA in Law 648/96 after CTS evaluation; no access for C and C(nn) drugs	
5 New Budget Law	€2bil yearly increase until 2025, +€600mln increase IDF until 2025, incremental increase of hospital drug cap	
6 AIFA Reform	Merging of CPR and CTS	
7 National Resilience and Recovery Plan	Strategic reforms such as strengthening territorial health, digital solutions and equity of access to care	

France	Detail	Areas
1 New Early Access System	ATU cohort system has been replaced with AAP	
2 ASMR IV eligible for inclusion on the liste en sus	National funding system for high-cost therapies, previously reserved for ASMR I-III	
3 Renegotiations for Price on ODs	If there is an increase in target population	
4 CEPS will consider the location of production	Intended to incentivize drug development in Europe and offer greater pricing stability	
5 HAS changes criteria for pharmacoeconomic evaluation	More drugs will require a cost-effectiveness model, particularly high budget drugs e.g., ATMPs	
6 Accelerated access for drugs with ASMR I-IV	Access to French market in 15 days, whilst a price is negotiated	

Spain	Detail	Areas
1 REvalMED	New network to coordinate IPT process	
2 HTA to include HE analysis	Manufacturers to provide info for HE analysis	
3 Increased use of OBAs	VALTERMED set up; 2-year training pilot for 750 clinicians	
4 ICER economic evaluation cap	ICER threshold expected	

A qualitative analysis has highlighted the most significant reforms



The reforms that should be monitored most closely

Germany
Reforms 1,2,4 — Cost containment policies could discourage manufacturers from launching ODs

France
Reforms 1,2,6 — Measures to increase speed of access could encourage OD launch and availability

Italy
Reforms 1,4 — Mounting pricing pressure and increased scrutiny of EAPs might add challenges to OD access

Spain
Reforms 1 — REvalMED should speed up the assessment process and shorten access times

England
Reforms 1 — Non-oncology ODs with clinical uncertainties will have better chance of access

Conclusion

Overall, attractiveness for OD launch is increasing in Europe. Due to the size of market potential, cost containment measures in Germany may hinder European launch strategy, mitigating positive impacts of increased OD patient access in other markets.

Monitoring upcoming changes and working closely with physician and patient advocacy groups to maintain OD funding priorities will ensure rare disease patients are not left behind and can continue to access new therapies