Unravelling the Relationship Between Expedited Regulatory Approval by the European Medicines Agency (EMA) and Reimbursement Outcomes for Drugs Approved under Exceptional Circumstances

HTA251

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INTRODUCTION

The European Medicines Agency (EMA) evaluate drugs' benefit/risk, while Health Technology Assessment (HTA) bodies assess added value for reimbursement. EMA may grant marketing authorization (MA) under "exceptional circumstances" when there is insufficient data on efficacy and safety due to disease rarity or ethical reasons.

OBJECTIVES

This study analyses HTA outcomes in Germany, Italy, France, and England for medicines approved under exceptional circumstances.

METHODS

- All products granted MA by EMA under exceptional circumstances between 2016-2020 were identified and assessed regarding inclusion criteria. Products approved later than 2020 were excluded as they are unlikely to have had time to undergo price and reimbursement procedures across markets.
- The EMA website and publicly available HTA resources in Germany, Italy, France, and England were searched to extract regulatory and HTA outcomes in May 2023.

RESULTS

- Between 2016-2020, 12 products were granted MA from the EMA under exceptional circumstances. Of the 12 products identified, 8 were included in the assessment, all of which had orphan designation (**Figure 1, Table 1**).
- Six products qualified for approval under exceptional circumstances "due to the **rarity of the disease**"; one due to both "the **rarity of the disease** and for **ethical reasons**."
- Number of reimbursed products per country: France (5), Italy (6), Germany (5), England (3) (**Table 2**).
- Average time to decision on reimbursement in years for these products was longer compared to the average of all drugs: France (4.1 vs. 1.4), Italy (2.7 vs. 1.2), England (2.1 vs. 0.9), and Germany (1.7 vs. 0.4) (**Figure 2**).
- For all products in France, Germany, and all except one in Italy, the reimbursed population was unrestricted versus the EMA-approved label. France required additional data, aligned with EMA requests, and Italy required a prescription registry for most reimbursed products. In England, reimbursed products underwent multiple review rounds, with two recommended for a subset of the population. All appraisals acknowledged caregiver/family burden's significance.

Figure 1. Process of inclusion

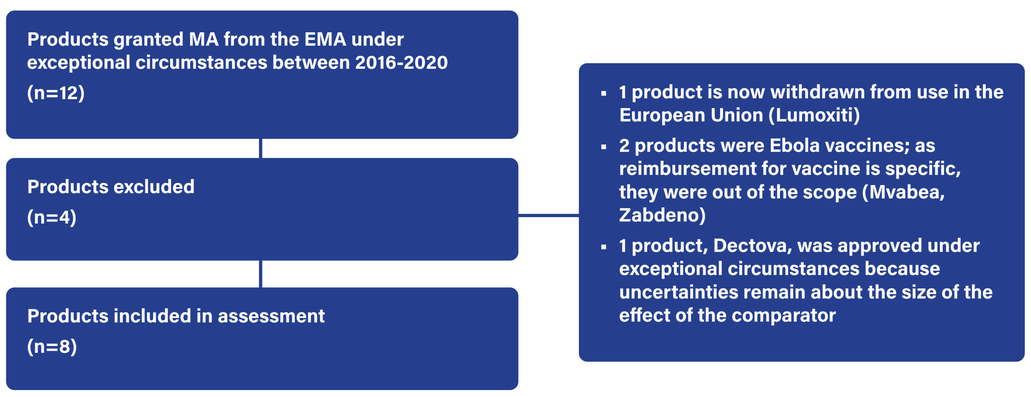


Table 1. EMA approval overview

Product	Indication	CHMP + Opinion	Therapeutic Area
Chenodeoxycholic acid[1]	Cerebrotendinous xanthomatosis	15 Sept 2016	Metabolism
Qarziba[2]	Neuroblastoma	23 March 2017	Cancer
Brineura[3]	Neuronal ceroid lipofuscinosis type 2 (CLN2)	21 April 2017	Neurology
Lamzede[4]	Alpha-mannosidosis	25 Jan 2018	Endocrinology
Mepsevii[5]	Mucopolysaccharidosis VII	28 June 2018	Endocrinology
Myalepta[6]	Generalised lipodystrophy, partial lipodystrophy	31 May 2018	Endocrinology
Elzonris[7]	Blastic plasmacytoid dendritic cell neoplasm (BPDCN)	12 Nov 2020	Haematology/ Haemostaseology
Nyxthracis[8]	Inhalational anthrax	17 Sept 2020	Infections

RESULTS

Table 2. Reimbursement status

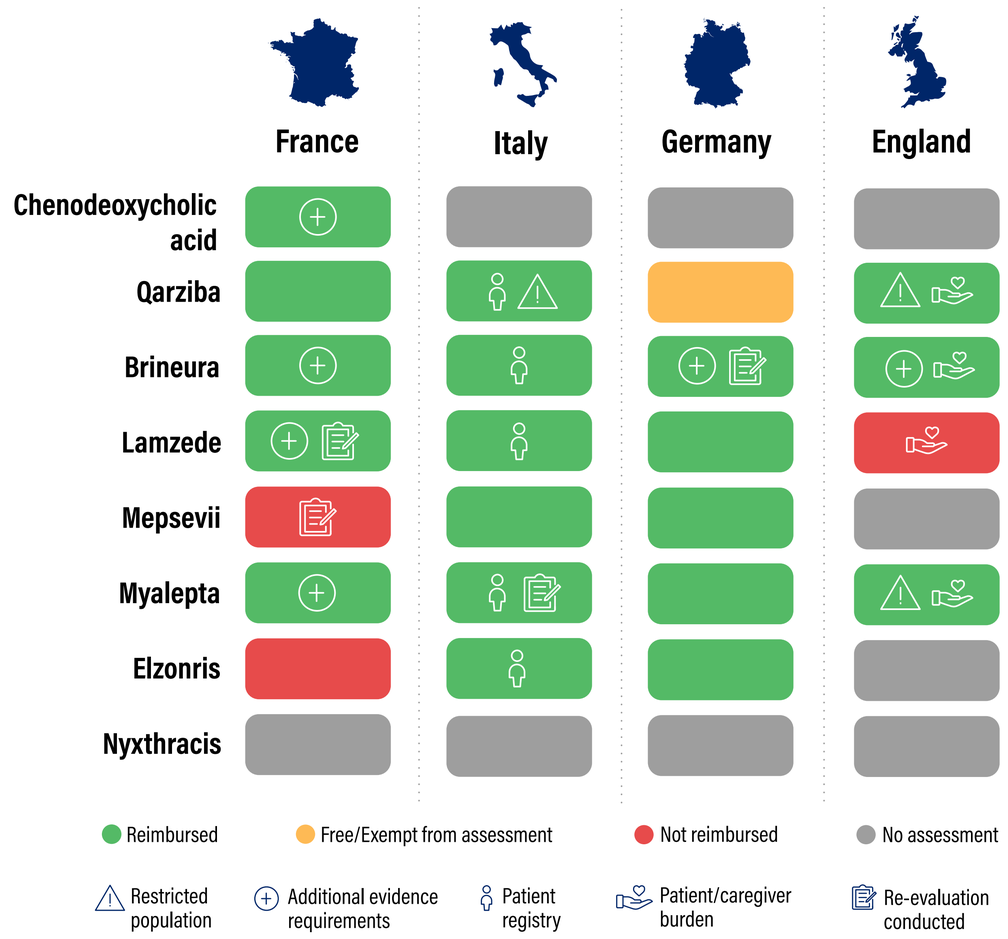
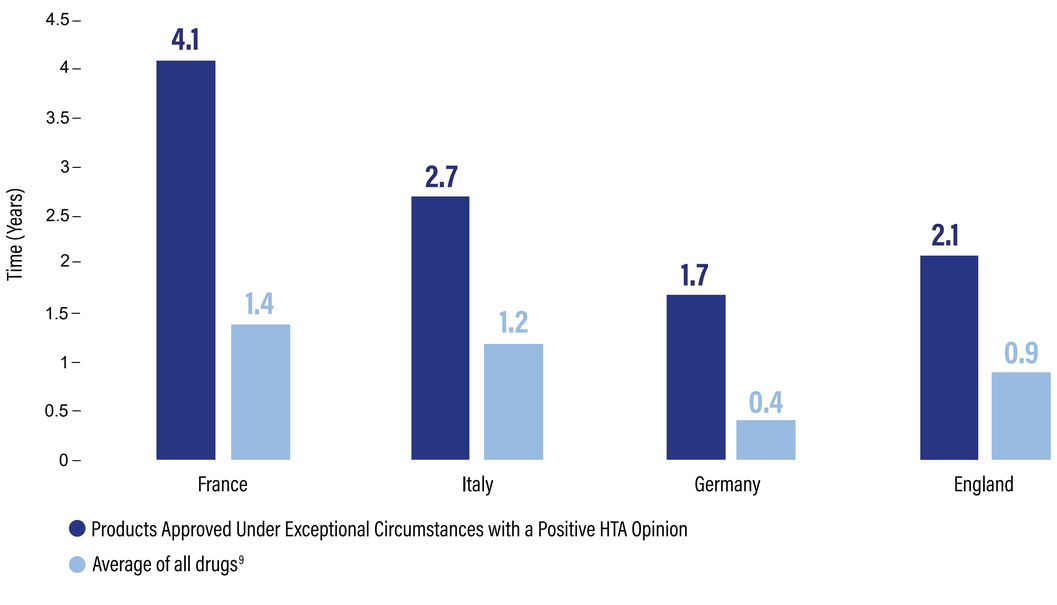


Figure 2. Time to reimbursement decision



CONCLUSIONS

Contrary to expectations, expedited regulatory approval under the exceptional circumstances pathway paradoxically results in a significant 2-5 times longer delay to pricing and reimbursement decisions, even for products with small patient populations and orphan status, compared to the average timeline of all drugs.

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