Impact of Expedited Regulatory Pathways on Reimbursed Access In EU4/UK/US (2020-2023)

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Objective

- To examine availability and market access timelines for medicines approved under expedited regulatory pathways from January 2020 to December 2023

Background

- Regulatory pathways exist to accelerate approval of medicines addressing unmet medical need, often based on less comprehensive evidence at time of authorization
- In Europe, the EMA may grant:
  - Conditional marketing authorization (CMA) when comprehensive data are expected to be generated post-approval
  - Approval under exceptional circumstances (EC) when comprehensive data cannot reasonably be obtained, even after authorization
- In the United States, multiple expedited review pathways including Fast Track, Breakthrough, Accelerated Approval, and Priority Review enable earlier approval based on limited or surrogate evidence
- While these pathways may shorten time to regulatory approval, their impact on reimbursed patient access remains unclear, particularly across European markets

Methods

- Identified 19 orphan medicines approved by the European Commission between January 2020 and December 2023 under conditional marketing authorization and exceptional circumstances
- Assessed whether corresponding products were approved in the US under expedited review pathways
- Evaluated market availability and time to standard reimbursed access and compared outcomes to all orphan drugs approved in the same period using the EFPIA W.A.I.T. indicator
  - Data was gathered from official national HTA agencies and P&R bodies; sources for launch date information provided in Table below

Table 1: Sources for launch dates

Country	Launch Date Information in the EU4, UK, and US
France	P&R decision (date published in Journal Officiel)
Germany	Product availability/introduction (ABDATA)
Italy	First P&R Decree publication on Official Gazette • Analysis of launch date does not consider initial approval in Class C-nn
Spain	Date of commercialization (Portalfarma)
UK	Product available and positive HTA recommendation (NICE/NHS)
US	Global Data

Results

Table 2: Drugs granted CMA by the EMA between Jan 2020 and Dec 2023

Brand	Generic (INN)	Disease state	EC approval date	Expedited US approval
Zolgensma	onasemnogene abeparvovec	Spinal muscular atrophy	18-May-20	✓
Idefirix	imlifidase	Kidney transplant desensitization	25-Aug-20	✓
Ayvakyt	avapritinib	Systemic mastocytosis	24-Sep-20	✓
Tecartus	brexucabtagene autoleucel	Mantle cell lymphoma	14-Dec-20	✓
Pemazyre	pemigatinib	Cholangiocarcinoma	26-Apr-21	✓
Koselugo	selumetinib	Neurofibromatosis type 1	17-Jun-21	✓
Minjuvi	tafasitamab	Diffuse large B-cell lymphoma	25-Aug-21	✓
Lunsumio	mosunetuzumab	Follicular lymphoma	3-Jun-22	✓
Roctavian	valoctocogene roxaparvovec	Hemophilia A	24-Aug-22	✓
Hemgenix	etranacogene dezaparvovec	Hemophilia B	20-Feb-23	✓
Talvey	Talquetamab	Multiple myeloma	21-Aug-23	✓

Table 3: Drugs approved under EC by the EMA between Jan 2020 and Dec 2023

Brand	Generic (INN)	Disease state	EC approval date	Expedited US approval
Elzonris	tagraxofusp	Blastic plasmacytoid dendritic cell neoplasm	1-Jul-21	✓
Bylvay	odevixibat	Progressive familial intrahepatic cholestasis	16-Jul-21	✓
Zokinvy	lonafarnib	Hutchinson-Gilford progeria syndrome	18-Jul-22	✓
Upstaza	eladocagene exuparvovec	Aromatic L-amino acid decarboxylase deficiency	18-Jul-22	
Voraxaze	glucarpidase	Methotrexate toxicity	11-Nov-22	
Livmarli	maralixibat	Alagille Syndrome	9-Dec-22	✓
Ebvallo	tabelecleucel	EBV+ post-transplant lymphoproliferative disease	16-Dec-22	
Loargys	arginine hydrochloride	Progressive familial intrahepatic cholestasis	15-Dec-23	

- 19 orphan medicines approved by the European Commission between 2020–2023 under conditional marketing authorization and exceptional circumstances
  - 84% (n=16) are new active substances
  - 74% (n=14) were also approved in the US under expedited regulatory pathways
  - 47% (n=9) have oncology indications
  - 32% (n=6) are ATMPs

Figure 1: Rate of availability (orphan drugs & drugs with CMA)

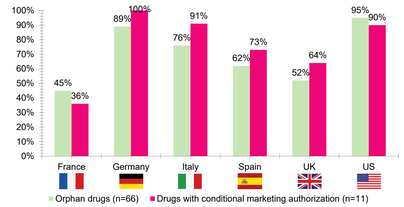
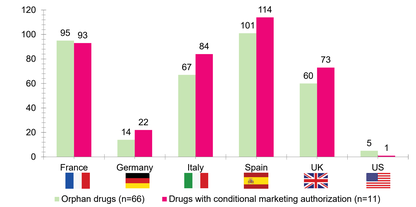


Figure 2: Time to availability (orphan drugs & drugs with CMA)



- Conditional marketing authorization (n=11):
  - All drugs approved under conditional marketing authorization completed P&R negotiations in Germany, 91% in Italy, 73% in Spain, 64% in the UK, and only 36% in France
  - Time to access was longer in most markets (DE, IT, SP, UK) by ~14 weeks compared to all orphan drugs approved during the same period
  - Only Zolgensma has converted from CMA to full standard marketing authorization; all other products remain conditional several years post approval

Figure 3: Rate of availability (orphan drugs & drugs approved under EC)

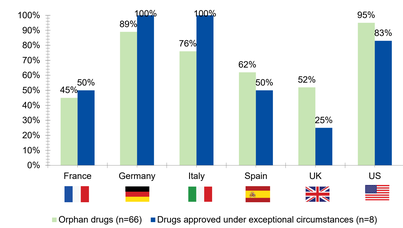
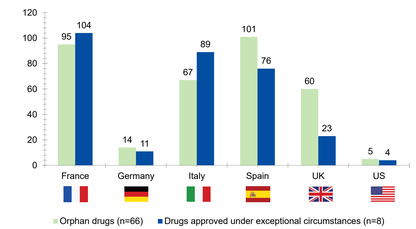


Figure 4: Time to availability (orphan drugs & drugs approved under EC)



- Exceptional circumstances (n=8):
  - While all drugs approved under exceptional circumstances had completed P&R negotiations in Germany and Italy, only 50% (n=4) were available in France and Spain and 25% (n=2) in the UK (non-submissions / terminated NICE appraisals)
  - For 2 approvals in the UK, access was substantially quicker (23 vs 60 weeks), while it was longer in Italy (89 vs 67 weeks)

Conclusions

- In the United States, multiple expedited review pathways including Fast Track, Breakthrough, Accelerated Approval, and Priority Review, to enable earlier approval based on limited or surrogate evidence
- In contrast, in Europe, medicines approved under exceptional circumstances face market availability challenges, and those with conditional marketing authorization experienced longer time to access
  - Conversion from CMA to standard MA was uncommon, suggesting that many products enter P&R negotiations under sustained evidence uncertainty
- Overall, regulatory acceleration alone is insufficient to ensure timely patient access in Europe, underscoring the need for stronger post-approval evidence and access planning alongside expedited approval

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