Squaring the Circle?
Leveraging Early Access / Compassionate Use Pathways to Provide Market-Specific Real-World Evidence before Launch

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Introduction

- Real-World Evidence (RWE) is an increasing focus of discussion to address limitations in clinical trial data
  - However, generation of RWE for a new therapy can only typically be generated after the product has launched
  - Nevertheless, payers want to see product-specific RWE at launch to inform reimbursement decision-making
- Early access / Compassionate Use programs (EAPs) offer the opportunity for patients to access therapies prior to marketing authorisation
  - This then offers the potential to generate RWE for a product in that market prior to its launch
- This research evaluates the EAPs available in major European countries to inform a discussion on whether these can be leveraged for RWE collection pre-launch

Methods

Publicly-available information was screened for information on EAPs in France, Germany, Italy, Spain, the UK, and the Netherlands using their respective websites and key information extracted.

Results

- All 6 countries offer EAPs *(Table 1)*, with many offering more than one (range: 1[Germany,Spain]–5[Italy]).
- Inclusion criteria is broadly aligned for most markets, in being for therapies in clinical development for severe/life-threatening diseases for patients with no other treatment options available
- Typically, applications can be made by either physicians or manufacturers although for some (e.g. Spain) it is physician-initiation only
- Reimbursement/charging incentives vary between countries and within available country programs
  - In 2/6 (Germany, UK) manufacturers must provide their treatments at zero cost
  - Another 2/6 (Spain, Netherlands) offer reimbursement only in select cases
  - Only 2/6 (Italy and France) offer full-reimbursement (although in Italy this is only in select programs).

Table 1: overview of EAPs across select European markets

Country	Agency	Scheme	Initiated by	Reimbursement offered?
	*BfarM/ PEI*	Ordinance on Medicinal Products for Compassionate Use (AHMV)	Manufacturer	No
	*HAS*	Early Access Authorisation (AAP)	Manufacturer	Yes
	ANSM	Compassionate Use Authorisation (AAC)	Physician	
		Compassionate Use Framework (CPC)	Ministry of Health	
	*AIFA*	Law 648/1996	Physician	Yes
		Compassionate Use (Law 94/98)		No
		AIFA National Fund (Law 326)		Yes
	*AEMPS*	Royal Decree 1015/2009	Physician	Generally No
	*MHRA*	Early Access to Medicines Scheme (EAMS)	Manufacturer	No
	*IGJ*	Named Patient Program	Physician	Generally No
	CBG	Compassionate Use Program	Manufacturer	

Conclusions

- All major European markets offer EAPs, potentially offering an avenue to collect product-specific RWE outcome data prior to its launch for reimbursement stakeholders
- However, there are additional challenges/barriers to this approach, including lack of reimbursement incentives, challenges in collecting outcomes data, and only severe patients being eligible who may experience worse clinical outcomes than the label population

Abbreviations: BfarM: German Federal Institute for Drugs and Medical Devices; PEI: Paul-Ehrlich Institute; HAS: French National Authority for Health; ANSM: French National Agency for the Safety of Medicines and Health Products; AIFA: Italian Medicines Agency; AEMPS: Spanish Agency of Medicines and Medical Device; MHRA: Medicines and Healthcare products Regulatory Agency; IGJ: Health and Youth Care Inspectorate; CBG: Medicines Evaluation Board

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