Orphan Drug Development Guidebook

Building Block E119

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	EMA - Initiative for Patient Registries
References	https://www.ema.europa.eu/en/human-regulatory/post-authorisation/patient-registries
Description	Patient registries are organised systems that use observational methods to collect uniform data on a population defined by a particular disease, condition, or exposure, and that is followed over time. Patient registries can play an important role in monitoring the safety of medicines. The European Medicines Agency (EMA) has set up an initiative to make better use of existing registries and facilitate the establishment of high-quality new registries if none provide an adequate source of post-authorisation data for regulatory decision-making: European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP). The inventory aims to facilitate the interaction between stakeholders and existing patient registries. Advice is to have early dialogue about PASS & PAES using registries, especially when multiple companies & products are being developed. Medicines for rare diseases are often authorized based on very limited data and further follow-up evidence (from registries) is a frequent regulatory request.
Category	Regulatory Building Block
Geographical	European Union

scope	
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	Non-interventional post-authorisation safety study (PASS) or efficacy study (PAES). Sponsor/MAH might be able to agree with EMA and registry holders to generate evidence from registries to support Benefit/Risk decisions for instance for indications for pediatric population, extensions of indication etc.
Stakeholders	• Pharmaceutical companies (sponsors & MAHs), • Registry coordinators, • Regulatory authorities (EMA, HTA, Inspectorate) • Developers
Enablers / Requirements	Qualification procedure of registries: Registries with good quality data Scientific Advice procedure on study protocols: Good registry studies (PASS & PAES) Study Team to do analysis
Output	Good quality data to support benefit/risk decisions. For instance good quality pharmacovigilance data.
Best time to apply and time window	The tool has its use during Scientific Advice at EMA and registry holders in contact with Sponsors.
Expert tips	Registries should be set up in well maintained and documented databases (not, for example, Excel files). Ensure registries are easy to use for those entering data into them; don't just think of ease of use for those who want the data out of them. PROs: • Data collection of current clinical practice.

	• MAHs are encouraged to design a joint registry study based on a single protocol CONs: • Financial support (often from one, or a few, commercial sponsors) and subsequent access to data by other stakeholders). • Ensuring long term viability (including financial support) for registries.