Orphan Drug Development Guidebook

Building Block E111

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	Potential for synergy between EU Orphan (Regulation (EC) No 141/2000) & Paediatric Regulation (Regulations (EC) No 1901/2006 and 1902/2006)
References	https://ec.europa.eu/health/human-use/orphan-medicines_en https://ec.europa.eu/health/human-use/paediatric-medicines_en https://www.ema.europa.eu/en/human-regulatory/marketing-authorisation/paediatric-medicines/paediatric-use-marketing-authorisations
Description	Each of these two regulations are laying down provisions for incentives and/or mandatory requirements to foster development of products for rare diseases and/or the paediatric population. Rare diseases and the paediatric population are often overlapping, and there is the potential for a synergistic effect of the available regulatory incentives - two additional years of Market exclusivity for products with an orphan designation having completed their Paediatric investigation plans (PIPs) by the time of the marketing authorisation submission. More than 50% of the Rare Diseases patients are less than 18 years old. Most paediatric diseases are rare.
Category	Regulatory Building Block
Geographical scope	European Union
Availability	Applicants developing medicines for rare and non-rare diseases. While engagement with the orphan regulation is voluntary, engagement with the paediatric regulation is mandatory for marketing authorisation (MA) approval.

Scope of use	Orphan designation can be requested early on in the development process, up until just before the filling of an application for MA. PIPs have to be completed before marketing authorization (with some exceptions in case of deferrals) but early engagement with the EMA's Paediatric Committee is also highly recommended Submit application to the EMA: https://www.ema.europa.eu/human-regulatory/research-development/orphan-designation/applying-orphan-designation https://www.ema.europa.eu/human-regulatory/research-development/paediatric-medicines/paediatric-investigation-plans/paediatric-investigation-plans-templates-forms-submission-dates
Stakeholders	EMA Committee for Orphan Medicinal Products (COMP) + EMA Paediatric Committee (PDCO)
Enablers/ Requirements	Having a product in development addressing a rare disease and intended for use in the paediatric population (+/- in adults)
Output	Benefits from the two legislations; 10 + 2 years of Market Exclusivity
Best time to apply and time window	As early as possible in the development process but with sufficient grounds to justify an orphan designation and ensure paediatric requirements can be discussed and fulfilled by the future development program.
Expert tips	https://www.eurordis.org/fr/publication/24th-workshop-eurordis-round-table-companies-bringing-solutions-young-rare-disease-patients PROs: • Synergistic effect of the two legislations offering additional incentives • There are no fees for applying for orphan designation or for engagement with the EMA's paediatric committee CONs: • Two types of dossiers and timelines from both procedures to be taken into account • PIP is a binding dossier to be prepared in CTD format and to be submitted to EMA through e-submission.