Orphan Drug Development Guidebook

Building Block E130

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	European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA)
References	https://www.ema.europa.eu/en/partners-networks/networks/european-network-paediatric-research-european-medicines-agency-enpr-ema
Description	The European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA) is a network of research networks, investigators and centres with recognised expertise in performing clinical studies in children. Enpr-EMA enables networking and collaboration with members from within and outside the European Union (EU), including academia and the pharmaceutical industry. It acts as a platform for sharing good practices as well as a pan-European voice for promoting research into medicines for children. More than 50% of the Rare Diseases patients are less than 18 years old. Most of the paediatric diseases are rare. Drug development in paediatric rare diseases will benefit from a more structured collaboration between developers and Paediatric Clinical Research Networks.
Category	Regulatory Building Block
Geographical scope	European Union

Availability	Networks, centres, or investigators, specialised in paediatrics that can support developing innovative medicines for rare and non-rare diseases.
Scope of use	Enpr-EMA members can offer pharmaceutical companies advice and expert information during all stages of paediatric medicine development. Enpr-EMA recommends, in particular, that medicine developers involve paediatric research networks when developing a paediatric investigation plan. Enp-EMA has published guidance for pharmaceutical companies on how to engage with paediatric research networks, including information on the services they offer. A fully searchable database provides easy access to each individual Enpr-EMA network: http://enprema.ema.europa.eu Enpr-EMA has published a **draft framework** for public consultation about **paediatric clinical trial preparedness**, with recommendations for sponsors, principal investigators and trialists: https://www.ema.europa.eu/en/documents/other/preparedness-medicines-clinical-trials-paediatrics-recommendations-enpr-ema-working-group-trial_en.pdf
Stakeholders	• EMA • Medicine developers
Enablers / Requirements	Enpr-EMA and EMA's micro, small and medium-sized enterprises (SMEs) office offer support in setting up partnerships between SMEs and academic investigators in paediatric medicine research, as these groups often experience difficulty finding partners that complement their research interests.
Output	Enpr-EMA's main objective is to facilitate studies in order to increase the availability of authorised medicines for children. It works by: – establishing a European paediatric research network of national and European specialist networks, investigators and centres with expertise in performing paediatric clinical trials to foster high-quality, ethical research on the safety and effectiveness of medicines for children; – efficient inter-network and stakeholder collaboration, to build up the necessary competences at European Union (EU) level, and to avoid unnecessary duplication of studies; – raising awareness among healthcare professionals, parents, carers, children and young people of the need and support for paediatric clinical trials; – assisting and entering into dialogue with ethics committees on issues relevant to

	research and clinical trials in children.
Best time to apply and time window	While setting up of Clinical trials.
Expert tips	PROs: – Faster access of medicine to patients