Drug Repurposing Guidebook

Building Block I458

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

ITEM	DESCRIPTION
Building Block (BB) Title	New formulation of drugs
References	http://www.eupfi.org/about-eupfi/
Description	European Paediatric Formulation Initiative (EuPFI) is a consortium working in a pre-competitive way on paediatric drug formulations. It aims to expedite the development of better and safer medicines for children by identifying and scoping issues and challenges in paediatric formulation development. It brings together the voluntary, academic, pharmaceutical industry, hospitals and regulatory agency in order to tackle the development of age-appropriate formulation for paediatrics
Category	Clinical development, including extrapolation of efficacy and safety data
Geographical scope	International
Availability	All stakeholders involved in pediatric formulation development
Scope of use	- Sharing expertise and interactive discussion between industry, academia, clinical and regulatory professionals. - Information dissemination and raising awareness (publications, conferences, etc.), linking and networking. - Identify the issues and challenges associated with development of pediatric formulation and consider ways

ITEM	DESCRIPTION
	towards better medications and clinically relevant dosage forms for children. - Promote early pharmaceutical consideration for the development of pediatric medicines. - Identify potential information, knowledge, know-how gaps in the paediatric formulation development. - Improve the availability of information of paediatric formulations.
Stakeholders involved	Members are from academia, hospital pharmacies, pharmaceutical industry (Innovators, Generics, Contract Research Organizations (CRO), Specials and Excipient Manufacturers) with European Medicine Agency (EMA) as an observer.
Enablers/ Requirements	Resources/expertise available to contribute to EUPFI and it workstreams.
Output	It provides funding, expertise and resources to support the solutions to the problems and new technologies emerging from academic research on age-appropriate formulation for paediatrics.
Best time to apply and time window	Membership is open to anyone interested in paediatric formulation development. Organizations/Institutions must apply for membership or sponsorship by filling the membership application form available on the website.
Expert tips	Some of their work could be related to the challenges of the RD patients as 70% of the RD have a pediatric onset. **Pros**: - work on the challenges in availability of and development of delivery devices for consistent administration of pediatric formulations. - age appropriateness of formulations.

ITEM	DESCRIPTION
	- provide guidance to industry and regulators on the use of Extemporaneous Preparations (EPs) and Industry Verified Preparations (IVPs) in the clinic. - focus on the limitations and technology gaps (in formulation platforms suitable for different age ranges) and identify opportunities more effective age-appropriate taste masking technologies. - pharmaceutical Excipients database project, known as STEP (Safety and Toxicity of Excipients for Pediatrics), designed to provide information for the risk assessment of use of excipients in children. - International collaboration with USA: EuPFI and USPFI are working together on the common project to build a database gathering safety data on excipients used in pediatric formulations. **Cons**: pediatric formulations only - IP situation of new formulations is unclear.