Orphan Drug Development Guidebook

Building Block I423

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	Feasibility-Patient engagement in trial endpoint selection
References	Consult general patient engagement and involvement tools and resources such as: https://eupati.eu/ https://imi-paradigm.eu/
Description	It is important that clinical trials measure disease, treatment and patient attributes that are relevant to patients. This is something that only patients can tell you firsthand. Methodologists can then assess whether there is an outcome measure available that can robustly and consistently measure this – if not, outcome measure(s) may need to be developed. Regulators will approve medicines based on the clinical benefit/risk profile – as such it is important that the trial endpoint measures something that is meaningful to patients. Health Authorities encourage patient input into the development of medicines. Having patient input around potential endpoints will be important, particularly where no regulatory precedence or pathway exists or where there is a lack of knowledge in the natural history of the condition.
Category	Development Practices Building Block

Geographical scope	International
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	To select primary and secondary endpoints for trials. Selecting trial endpoints that are relevant to patients will help ensure that the results of the trial (if positive) are acceptable to the regulators, healthcare professionals and the patient community
Stakeholders	• Patients and Drug developers • Regulators (including payers) to confirm if selected endpoint is regulatory compliant
Enablers/ Requirements	The sponsor of the trial should initiate this initiative
Output	Better trial design
Best time to apply and time window	The tool has its best use between First-in-human ready and before Pivotal data.
Expert tips	Involve patient representatives that are aware of the regulatory system and able to provide pan-community representation on the needs of their patient group. PROs: – Inclusion of endpoints that are relevant to the patients CONs: – The patients involved need to either be aware of the needs of their community, or multiple patients need to be involved, to avoid tailoring the endpoint selection to the wishes of one patient – Patients involved need to be aware of regulatory and drug development issues (e.g. by attending EURORDIS summer school or EUPATI alumni)
