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	
Description	It is important that pivotal clinical trials measure something that is relevant to patients. This is something that only patients can tell you. Academics can then assess whether there is an outcome measure available that can 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 development of medicines. Having patient input around potential endpoints will be important, particularly where no regulatory precedence or pathway exists.
Category	Development Practices Building Block
Geographical scope	International

Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	To select primary endpoints for pivotal trials. Selecting a trial endpoint that is relevant to patients will help ensure that the results of the trial (if positive) are acceptable to the regulators
Stakeholders	• Patients and Drug developers • Regulators 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. PROs: – An endpoint that is 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 the regulatory issues (e.g. EURORDIS summer school or EUPATI alumni)