

Orphan Drug Development Guidebook

Building Block I422

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 design and feasibility
References	<p>Eurordis as first point of contact; the patient organization of the specific disease</p> <p>https://www.eurordis.org</p> <p>https://www.eupati.eu</p>
Description	<p>It is important to involve patients at an early stage of designing clinical trials. This will ensure the trial is better tailored to patient needs. Specifically, patient input can help ensure that the burden of the trial is acceptable to and feasible by patients (e.g. with regards to accessibility issues, fatigue, assessments) but also ensuring inclusion criteria do not exclude all patients). Patient input is also important to confirm that trials are measuring what matters to people living with the condition. This is especially the case for diseases with no clinical trial history or no regulatory pathway.</p>
Category	Development Practices Building Block
Geographical scope	International
Availability	Applicants developing medicines for rare diseases.
Scope of	Involving patients may potentially expedite recruitment, support retention and enhance

use	<p>execution of clinical trials and potentially avoid trial amendments. Involving patients in trial design will likely increase compliance and will prevent designs that are unrealistic or with too high a patient or caregiver burden, or to design a trial that makes it impossible to recruit the required cohort.</p> <p>Patient input can also be helpful in operationalization aspects of the trial, eg input into informed consent process and the ICF, supportive documentation, material and communication.</p>
Stakeholders	<ul style="list-style-type: none"> • Patient representatives • Drug developer of rare and non-rare disease
Enablers / Requirements	None
Output	More patient-centric trial design and easier/potentially faster recruitment and study completion
Best time to apply and time window	The tool has its best use between First-in-human ready and before Pivotal data.
Expert tips	<ul style="list-style-type: none"> – When doing this, the conflict / declaration of interest of patients needs to be taken into account (participating in this effort may preclude them from participating as patient experts in scientific advice to the regulators) – Ensure sufficient time is built in to the process to allow for quality input – Provide feedback to patients about the impact of their input <p>PROs:</p> <ul style="list-style-type: none"> – This avoids the set-up of trial with a high burden or designs that are not feasible for patients (accessibility, physical tests, caregiver impact, inclusion criteria etc) – Health Authorities encourage greater patient involvement in drug development <p>CONs:</p>

	<ul style="list-style-type: none">– One patient cannot be representative of the whole patient community – ideally multiple patients, or sources of patient input, are involved, e.g.: a CAB, or patient representatives who can reflect the needs of their community– Adequate time should be factored into the clinical trial development process to allow for patient input.
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