Orphan Drug Development Guidebook

Building Block I431

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	Target Patient Value Profile
References	https://www.eurordis.org/content/eurordis-community-advisory-board-cab-programme https://www.fda.gov/drugs/development-approval-process-drugs/external-resources-or-information-related-patients-experience https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials https://rarediseases.info.nih.gov/toolkit/home https://patientfocusedmedicine.org/
Description	A document outlining the goals, profile and potential benefit of a specific product, addressing relevant current and future patient needs in a differentiated way. It provides accurate, up-to-date information describing the expected benefit for patients and describe the drug.
Category	Development Resources Building Block
Geographical scope	International
Availability	This tool is developed for the benefit of drug developers.

ITEM	DESCRIPTION
Scope of use	Guides product discovery and development towards the meaningful benefit and needs expressed by patients, and can also be used as a communication tool to patients and investigators and regulatory authorities to frame the potential value of the product in development from the patient perspective. As guide and starting point to define the product development plan.
Stakeholders	Drug developers, patients
Enablers/ Requirements	Access to patients, likely through advocacy groups
Output	Patient-centric information and resources
Best time to apply and time window	Best to access early on in development to get full view of research and activities in the disease-specific space. Prepared first at the beginning of development, and periodically reviewed.
Expert tips	Best developed at the very start of drug development. **PROs**: Comprehensive information about patient group for which the drug will be developed. **CONs**: None