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.ninds.nih.gov/current-research/research-funded-ninds/translational-research/create-bio/create-bio-application-support-library/create-bio-example-target-product-profile-tpp https://www.gov.uk/guidance/the-target-development-profile-toolkit
Description	A document or a part of 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. Inspiration comes from the US FDA Target Product Profile which is a planning tool for therapeutic candidates based on FDA guidelines. Similar approach is also present in the UK ILAP scheme with the Target Development Profile (see links in the Reference section)
Category	Development Resources Building Block
Geographical scope	International
Availability	This tool is developed for the benefit of drug developers.
Scope of use	It guides product discovery and development towards the meaningful benefit and needs expressed by patients, and can also be used as a

ITEM	DESCRIPTION
	communication tool to patients, investigators and regulatory authorities to frame the potential value of the product in development from the patient perspective. A guide and starting point to define the product development plan.
Stakeholders	Drug developers, patients
Enablers/ Requirements	Involvement of patients/patient advocacy groups
Output	Patient-centric information and resources / patient-centric development plan
Best time to apply and time window	Early on ie during the development planning phase to get full perspective of patient needs 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 needs steering the product development plan. **CONs**: None