Orphan Drug Development Guidebook

Building Block U217

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	Stakeholder interaction – Professional affairs and Community Engagement
References	Example: https://www.researchgate.net/publication/303831422_Stakeholder_cooperation_to_overcome_challenges_in_orphan_medicine_development_The_example_of_Duchenne_muscular_dystrophy The Express EURORDIS summer school (patients and academics are pupils and are educated about drug development and the regulatory system).
Description	There are different stakeholders in drug development: patients, academics, industry, regulators and payers. For rare diseases with no available therapy often multi-lateral education is required: regulators and payers are not aware of the current state of the art of that specific disease research, or of the impact and burden of the disease on daily life of the patients. At the same time academics and patients are often not aware of the way the regulatory system works. Initiating the dialogue between these stakeholders is best done not for a specific drug, but for a specific disease or disease groups. Interaction of the stakeholders and multilateral education at an early stage of development will facilitate drug development (e.g. ensuring outcome measures for clinical trials are developed that are meaningful for patients, are acceptable to the regulators, and are acceptable to payers).
Category	Regulatory Building Block

Geographical scope	United States of America
Availability	Applicants developing medicines for rare diseases.
Scope of use	Early interaction between stakeholders will ensure outcome measures are developed that measure what is important to patients and are acceptable to regulators and payers
Stakeholders	• Academics: develop relevant and regulatory compliant outcome measures, prepare for gaps in knowledge/tools for regulatory approval • Regulators: learn about the state of the art of a specific rare disease and learn about the patients' need • Patients: relevant outcome measures, smoother drug development process • Payers: learn about the patients' needs and burden (cost) of the disease
Enablers / Requirements	Someone needs to initiate the dialogue (generally patients or academics).
Output	More aware stakeholders and better outcome measures.
Best time to apply and time window	The regulators will not have time to have a dialogue for each rare disease all at once. However, when preclinical work looks promising it is best to start a dialogue to avoid the set-up of suboptimal trials. Companies should get educated on the disease burden and patient experience before designing clinical trials.
Expert tips	DO: • Work within the patient community to have an open dialogue with companies and regulators about disease experience and disease burden.

	• Identify if clinical outcomes are suitable, and if these are not available collaborate with academics and industry in developing clinical outcome measures before clinical trials start. • Seek advice on how to collaborate with the industry and learn to see them as a partner. DON'T: • Rely only on academics to help you design clinical outcomes and other important elements of drug development. These are specific science fields that require industry and regulators feedback and often collaboration.