Orphan Drug Development Guidebook

Building Block I414

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	Patient surveys / Patient Preferences studies / Ethnographic research
References	https://www.imi-prefer.eu https://www.fda.gov/media/92593/download
Description	Qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions. Patient preference studies may be particularly useful in evaluating a drug's benefit-risk profile when patient decisions are "preference sensitive." Patient decisions regarding treatment options are preference sensitive when: 1) multiple treatment options exist and there is no option that is clearly superior for all patients; 2) when the evidence supporting one option over others is considerably uncertain or variable; and/or 3) patients' views about the most important benefits and acceptable risks of a drug vary considerably within a population, or differ from those of healthcare professionals.
Category	Development Practices Building Block

Geographical scope	International
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	Patient preference studies can be useful during regulatory benefit-risk assessment for certain drugs in several major ways, including: 1) to help identify the most important benefits and risks of a drug from a patient's perspective (including to inform selection of primary or secondary endpoints); 2) to assess the relative importance to patients of different attributes of benefit and risk, and clarify how patients think about the trade-offs of these benefits and risks for a given drugs (including to inform minimum clinically important benefit and effect size); 3) to help understand the heterogeneity or distribution of patient preferences regarding benefits and risks of various treatment options (including to inform patient subgroup considerations as part of benefit-risk assessments). The specific role of quantitative patient preference studies are to provide estimates of how much different outcomes, endpoints or other attributes are valued by patients, and the tradeoffs that patients state or demonstrate they are willing to make among them.
Stakeholders	• EMA, FDA and/or MHLW • Drug developers • Patient organisations • Reimbursement agencies
Enablers / Requirements	Early contact with patient organizations.
Output	A report on the outcomes of the preferences of patients regarding treatment options, that can be used for different purposes, such as regulatory or payer's assessment.
Best time to apply and time	The tool may be used at several stages during drug development, therefore an early development is recommended.

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Expert tips	Patient preference studies may not be relevant or appropriate for all drug types. Submission of patient preference studies to regulatory agencies is voluntary.