Orphan Drug Development Guidebook

Building Block I416

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	Companion Diagnostics
References	https://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM262327.pdf https://www.ema.europa.eu/en/human-regulatory/overview/medical-devices#companion-diagnostics-('in-vitro-diagnostics')-section
Description	A companion diagnostic is a medical device, often an in vitro device, which provides information that is essential for the safe and effective use of a corresponding companion drug or biological product. The test helps a health care professional determine whether a particular therapeutic product's benefits to patients will outweigh any potential serious side effects or risks. Companion diagnostics can: – identify patients who are most likely to benefit from a particular therapeutic product; – identify patients likely to be at increased risk for serious side effects as a result of treatment with a particular therapeutic product; or – monitor response to treatment with a particular therapeutic product for the purpose of adjusting treatment to achieve improved safety or effectiveness.
Category	Development Practices Building Block

Geographical scope	International
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	Drug developers might develop drugs for a specific subtype of patients defined by particular genetic or molecular features. Drugs in development that are intended to be used in a biomarker-defined subtype of patients may require a companion diagnostic. Companion diagnostics are tests that provide information essential for the safe and effective use of a corresponding drug. Therefore, if the drug is likely to only have a favorable benefit-risk profile in a biomarker-defined subtype of patients, the drug developer should consider developing a companion diagnostic early in the development.
Stakeholders	• Drug developers • FDA • EMA
Enablers/ Requirements	Not applicable
Output	Diagnostic Tool
Best time to apply and time window	The tool has its best use at the early phases of development.
Expert tips	Companion diagnostics enable personalised medicine by analysing a patient's DNA to determine if their genetics are a match for a given drug. In most circumstances, if use of an in vitro companion diagnostic device is essential for the safe and effective use of a therapeutic product, the companion diagnostic device and the therapeutic product are approved or cleared contemporaneously for the use indicated in the therapeutic product labelling by FDA/ EMA.