Orphan Drug Development Guidebook

Building Block E117

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	Business Pipeline Meetings
References	https://www.ema.europa.eu/documents/leaflet/business-pipeline_en.pdf https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/support-applications-article-58_en.pdf
Description	Business pipeline activity is about anticipating in a timely manner the quantitative and qualitative impact of pharmaceutical pipelines on the operations of the European Medicines Agency (EMA). The business pipeline activity is a tool for accurate budgeting and identification of the most appropriate resources and scientific expertise. Business pipeline meetings are instrumental in contributing to EMA's preparedness, as well as helping the drug developer to identify issues/resources at an early stage. Business pipeline meetings can be used to discuss orphan designation and extensions, marketing-authorisation application (MAA), and paediatric investigation plan (PIP), scientific advice, etc., in the context of orphan drug development. As an early-stage dialogue, business pipeline meetings are short discussions between drug developers and EMA. It also takes minimum amount of time to apply/initiate.
Category	Regulatory Building Block

Geographical scope	European Union
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	Business pipeline meeting is a confidential and mutually beneficial discussion on the drug developer's products' regulatory pipeline, which includes for example, marketing- authorisation application (MAA), paediatric investigation plan (PIP), scientific advice, orphan designation and extensions. The focus is on general aspects of the product portfolio, while specific scientific or regulatory issues can be discussed at the scientific advice stage, or in the pre-submission meetings for the marketing authorisation, paediatric investigation plan and orphan designation. The primary goal is to identify at an early stage any issues impacting the progress of your product portfolio, and to effectively anticipate scientific expertise needed, guideline discussion, changes in technology and drug development.
Stakeholders	• Drug Developers • EMA
Enablers/ Requirements	Drug developers with an R&D drugs pipeline.
Output	Provides horizon-scanning analysis to EMA's scientific committees.
Best time to apply and time window	These meetings are best to happen at early stage of development (see relevant guidance from EMA links above).
Expert tips	Use this type of meetings to identify at an early stage issues impacting the progress of your product portfolio, and to effectively anticipate scientific expertise needed, guideline discussion, changes in technology and drug development. PROs: – A unique opportunity to establish a confidential and mutually beneficial discussion on the drug developer's products' regulatory pipeline, e.g.

	marketing- authorisation application (MAA), paediatric investigation plan (PIP), scientific advice, orphan designation and extensions.