Orphan Drug Development Guidebook

Building Block E121

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	European Network for Health Technology Assessment (EUNetHTA)
References	https://www.eunethta.eu/
Description	EUnetHTA was established to create an effective and sustainable network for HTA across Europe – we work together to help develop reliable, timely, transparent, and transferable information to contribute to HTA in European countries. EUnetHTA supports collaboration between European HTA organizations that brings value at the European, national, and regional level through: • The facilitation of efficient HTA resource use. • The creation of a sustainable system of HTA knowledge sharing. • The promotion of good practice in HTA methods and processes. The procedure has a duration of 4.5 months.
Category	HTA and reimbursement Building Block
Geographical scope	European Union

Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	EUnetHTA provides advancing cooperation on health technology assessment (HTA) and supporting the implementation of the new EU legal framework on HTA EUnetHTA provides a non-binding scientific advice, before the start of pivotal clinical development in order to improve the quality and appropriateness of the data produced by the developers in view of future HTA assessment / re-assessment. EUnetHTA early dialogues should enable to exchange between the applicant and these agencies at an early stage in the development process in order to allow for the integration of HTA requirements (e.g. choice of comparators, relevant outcomes, quality of life, patient groups) in the study design (pivotal trials & post-launch studies) and the economic evidence generation plan. The main objective of EUnetHTA early dialogues is to gather and provide the common recommendations on how the drug or device could be developed in order to fill HTA requirements across multiple European Member States. However, when consensus is not possible, the views of participating HTA bodies will be made known to the applicant. EUnetHTA provide also a series of guidelines (EUnetHTA Methodology Guidelines) focused on methdological challenges that are encountered by HTA assessors while performing relative effectiveness assessments of pharmaceuticals or non-pharmaceutical health technologies.
Stakeholders	• Developers; • HTA bodies; • Patients
Enablers/ Requirements	The request for a EUnetHTA ED must be made in the form of a Letter of Intent. The Letter of Intent is specific to the procedure and the company applying for an ED should use the appropriate template.
Output	Set of non-binding recommendations
Best time to apply and time window	The tool has its use after feasibility / proof of concept studies.

Expert tips	PROs: – Allows a drug developer to receive advice from multiple HTA bodies at the same time CONs: – Joint Assessment on your suggested compound will only be performed if sufficient EUnetHTA partners are interested in working on this topic, since one of the aims of EUnetHTA is to ensure high uptake of EUnetHTA assessments in European Member States