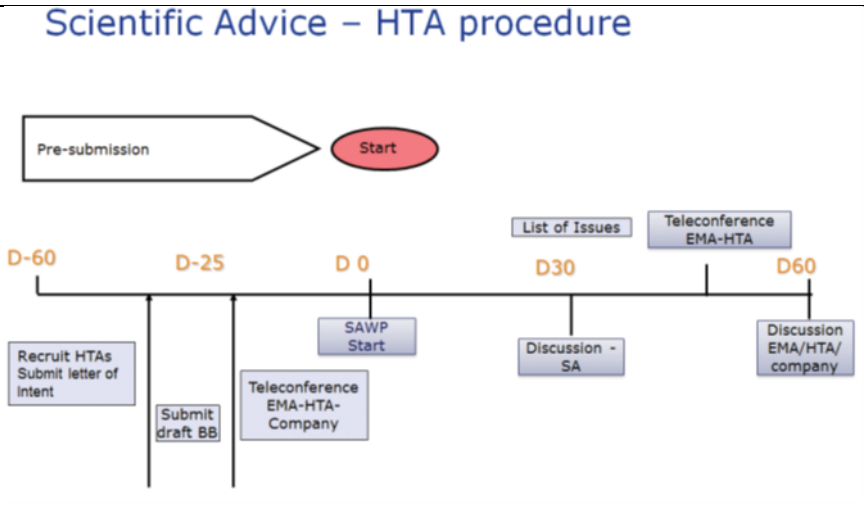
Orphan Drug Development Guidebook

Building Block E134

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	Joint EMA-HTA Scientific Advice
References	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_001857.jsp&mid=WC0b01ac0580a11c96
Description	The European Medicines Agency (EMA) offers consultations in parallel with the European Network for Health Technology Assessment (EUnetHTA) 21 consortium, as of 2022. This aims to allow medicine developers to obtain feedback from regulators and health technology assessment (HTA) bodies on their evidence-generation plans to support decision-making on marketing authorisation and reimbursement of new medicines at the same time. The BB is applicable to all types of medicinal products but may be of special relevance to avoid delays in access and because of the need of equity in the access to orphan medicinal products across EU; because of that it is key to ensure that convergence in the assessment of value is approached in an early manner. The procedure duration is the following:

	 The procedure has four stages: submission of request to EMA via IRIS and briefing package to EUnetHTA21 simultaneously, pre-submission, evaluation and advice/outcome. EMA and EUnetHTA have published joint guidance for the parallel consultation procedure which explains how to apply and highlights the actions for each party and preferable timelines: http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2017/07/WC500230375.pdf
Category	Regulatory Building Block
Geographical scope	European Union
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	To allow medicine developers to obtain feedback from regulators and health technology assessment (HTA) bodies on their evidence-generation plans to support decision-making on marketing authorisation and reimbursement of new medicines at the same time.
Stakeholders	• EMA • European Network for Health Technology Assessment (EUnetHTA) • Drug developers applying for market authorization

Enablers / Requirements	To benefit from the procedure, the applicant must first, submit a letter of intent to the EMA and the EUnetHTA simultaneously.
Output	The objective is to help generate optimal and robust evidence that satisfies the needs of both regulators and HTA bodies.
Best time to apply and time window	The tool can be used starting from product discovery until market access being the optimal times to apply right before First in Human Ready, after human PoC and before market authorization.
Expert tips	Companies may apply for advice at different times of development. Often applicants wait until they have clinical data and when they are planning late Phase II and Phase III clinical studies; at this time, they can receive responses on study design, duration, populations for inclusion, comparators and endpoints, and also receive input on data to be gathered in order to support later the cost-effectiveness and added therapeutic value of the product. PROs: The main benefits of the parallel consultation procedure include: • Streamlined procedure for applicants; • Increased mutual understanding and problem-solving ability between EMA and HTA bodies through a more structured interaction; • Improved coordination with, and greater participation of HTA bodies in parallel consultations through EUnetHTA's Early Dialogue Working Party (EDWP) and the EUnetHTA early dialogue (ED) secretariat. The EUnetHTA ED secretariat facilitates the centralised recruitment of HTA bodies. Patient representatives and healthcare professionals also participate in the parallel consultation procedure on a routine basis so that their views and experiences are incorporated into discussions. CONs: The differences in standards of care and type of healthcare systems across EU may be difficult to reconcile at the time of setting the degree of priority of interventions, and consequently of value. Since the procedure may not be binding, in case of substantial divergence in criteria the results of the procedure may not be predictive of the final outcome of HTA assessment and access.