Orphan Drug Development Guidebook

Building Block E120

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	Mechanism of Coordinated Access to OMPs (MoCA)
References	https://www.eurordis.org/content/moca
Description	Early dialogue with payers /Medev - MoCA provides a mechanism for European countries to collaborate on coordinated access to orphan medicines in a voluntary, dialogue-based approach, intended to create a fluid set of interactions between key stakeholders, across all aspects of a product. The roles, responsibilities and prerogatives of each of the participating stakeholders should be respected. It is important to stress that, to date, nowhere else in Europe does such a platform exist where companies' issues around reimbursement or financing schemes can be discussed with such a variety of jurisdictions and societal perspectives.
Category	HTA and Reimbursement Building Block
Geographical scope	European Union
Availability	Applicants developing medicines for rare diseases.

Scope of use	During the development, in early phases, to shape the development with the payers view in mind so to align with their requirements. Platform to discuss the acceptability/feasibility of reimbursement schemes. Opportunity to fill in the Transparent Value Framework, an instrument useful to assign value as it lists important criteria contributing to the value of an orphan medicinal product. Apply the advice to the development design and/or reimbursement strategy, in order to prepare the future discussions with NCA for pricing and reimbursement at the time of market entry.
Stakeholders	Participation in MoCA is open to several stakeholder groups, including: • National competent authorities for pricing and reimbursement • Rare disease patients • Candidate marketing authorisation applicant/holders willing to be involved in a pilot focused on a particular product of theirs.
Enablers / Requirements	Put a request forward by contacting MoCA Steering Group via: **Anna Bucsics**, MoCA Project Advisor Šárka Kubinová, MoCA Project Coordinator MoCA.OMP@gmail.com **Maria Cavaller**, Patient Engagement & Therapeutic Development Senior Manager maria.cavaller@eurordis.org
Output	Iterative process with several discussion meetings with a group of EU reimbursement agencies/payers representatives – Recommendations to shape the development according to payers' requirements. No binding agreement.
Best time to apply and time window	The tool has its use as early as possible in the development process but also possible to apply later in the development phase, including post-approval.
Expert tips	Coordinated access to orphan medicines: using a dialogue-based approach between key stakeholders (Simone Boselli, Anna Bucsics, Wills Hughes-Wilson): http://download2.eurordis.org.s3-eu-west-1.amazonaws.com/moca/presentations/MoCA_EuropaBio_2018.pdf Update on MoCA: https://download2.eurordis.org/moca/presentations/PRES-2019-Update-on-MoCA.pdf

	PROs: – Early alignment, identification of the needs pertaining to the national situations – the voice of patients is embedded into these discussions as EURORDIS is identifying patient experts to be invited to contribute to the meetings. CONs: – Non- binding and not all the payers are represented as attendance is on voluntary basis