Drug Repurposing Guidebook

Building Block I461

This document defines the content of the FACT SHEET to be created for each identified tool, incentives, initiative or practice (the Building Block) introduced by public bodies or used by developers to expedite drug repurposing in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	How to develop pricing models for repurposing
References	EU: EUnetHTA; EC - Public Health – Health Technology Assessment; HTA Regulation; MoCA (Mechanism of Coordinated Access to Orphan Medicinal Products) UK: NICE USA: ICER International: HTAi
Description	Plan for collecting evidence to support calculation and negotiation of the price for the re-purposed product
Category	Regulatory and HTA engagement
Type of BB	HTA and reimbursement
Geographical scope	International
Availability	Applicants re-purposing medicines for rare diseases, HTA bodies and payers
Scope of use	This BB is used to evaluate market landscape, calculate cost of drug development, incorporate risk-benefit assessment and develop the

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	general pricing model that could be adjusted to fit for a specific market
Stakeholders involved	• Drug developers • Health Technology Assessment (HTA) organizations • Payers • Investment funds • Patients' organisations • Regulatory agencies • Healthcare professionals
Enablers/ Requirements	Risk-benefit assessment based on the evidence of drug efficacy and confirmation of drug safety Cost of drug development Price of competitive product(s) Price of originator product
Output	Pricing
Best time to apply and time window	The tool should be used through the clinical development: • At the Phase 1/ 2 studies it is used to identify value of the product. • At the Phase 3 study it is used to create value of the product through evidence of its efficacy. It is important to have meeting(s) with HTA organizations and with national authorities for pricing and reimbursement to seek advice and reach an agreement.
Expert tips	The pricing model development should start together with the clinical development and it should be re-adjusted based on the value of the approved drug and the patients' needs. Developers should request early advice to HTA experts on what data is needed, to have this trajectory as smooth as possible.