Drug Repurposing Guidebook

Building Block E139

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	EMA pilot project to support academia
References	• EMA-website: https://www.ema.europa.eu/en/news/repurposing-authorised-medicines-pilot-support-not-profit-organisations-academia including the following documents: • Questions and Answers on repurposing pilot project on proposal for framework to support not-for-profit organisations and academia in repurposing authorized medicines. • Submission form – Repurposing pilot project for authorised medicines
Description	The medicines repurposing framework proposal was developed by the European Commission's STAMP Expert Group composed of representatives of EU Member States together with EMA and stakeholders from not-for-profit organisations, patients, healthcare professionals, industry, health technology assessment bodies and payers. EMA and the Heads of Medicines Agencies (HMA) launched in October 2021 a pilot project to support the repurposing of medicines as a follow-up to the European Commission's Expert Group on Safe and Timely Access to Medicines for Patients (STAMP) discussions on a proposal for a medicine repurposing framework. The aim of the pilot is to support not-for-profit organisations and academia (only) to gather or generate sufficient evidence on the use of an established medicine in a new indication with the view to have

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	this new use formally authorised by a regulatory authority. This project doesn't provide grants/funds, instead it meant to provide indirect support through advices on how to generate robust and reliable data packages. The innovation of the project is that in case of selection, then the project is not receiving a single scientific advice, but instead a series of advices until a marketing licensee who can bring the product to the registration/market is identified. The EMA fees for a scientific advice are free in case of orphan, otherwise are those payable to the EMA (https://www.ema.europa.eu/en/human-regulatory/overview/fees-payable-european-medicines-agency). The pilot will run until the completion of scientific advice for the selected repurposing candidate projects and optimally until the filing of an application by a pharmaceutical company for the new indication. A report will be published after the pilot. Repurposing of medicines for COVID-19 falls outside the scope of this pilot project.
Category	Regulatory and HTA engagement
Type of BB	Regulatory
Geographical scope	Europe
Availability	Only for Not-for-profit organizations and academia (institutions and individuals) who has a particular interest in repurposing an authorized medicine for a new indication in an area of public health interest, have a rationale for their repurposing program and would like to seek scientific advice from a regulatory authority.
Scope of use	EMA proposes to support the development and implementation of a repurposing framework in its Regulatory Science Strategy to 2025, which is its plan for advancing engagement with regulatory science over the next five to ten years. While marketing authorization holders may develop medicines for uses in other indications, sometimes they lack the incentives or the commercial interest to pursue the necessary research and development and complete the regulatory process needed for the authorization of a new indication for old medicines which are no

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	longer protected by a patent or data exclusivity. This could be a wasted opportunity for public health. At the same time, academic institutions and/or patient organizations may be interested in carrying out this development for the benefit of public health. However, they may not have the necessary regulatory experience and have no intention of becoming a marketing authorization holder themselves.
Stakeholders involved	Not-for-profit organisations, academia, industry developing repurposed drugs and regulators
Enablers/ Requirements	The deadline for submission for projects was 28 February 2022. Currently, it is not possible to participate anymore in this pilot. For information, candidate medicines for the pilot should have fulfilled the following criteria: - contain a well-established active substance; - be an authorised medicine (containing the concerned active substance) out of data exclusivity and market protection periods and out of basic patent / supplementary protection certificate (SPC) protection; - target an indication in a condition distinct from the currently authorised indication(s); - target an indication in an area where important public health benefits are likely to be achieved. Conditions for which no or few medicines are currently authorised or which are associated with high morbidity and / or mortality despite available medicines, will be the focus of the pilot.
Output	A report will be published after the pilot project is completed, i.e. when all selected drug repurposing projects have received scientific advices from the EMA and/or national agencies and optimally until the filing of an application by a pharmaceutical company for a new indication.
Best time to apply and time window	Not applicable, as currently it is not possible to apply anymore for this pilot. EMA website to be checked periodically in case another pilot project is to be implemented.

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Expert tips	Not applicable (the pilot is currently running; no experience tips to be shared right now).