Drug Repurposing Guidebook

Building Block E136

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	DARWIN-EU (data-analysis-real-world-interrogation network)
References	EMA-Website: https://www.ema.europa.eu/en/about-us/how-we-work/big-data/data-analysis-real-world-interrogation-network-darwin-eu
Description	DARWIN-EU is a new European initiative with the aim to give EMA and national competent authorities in EU Member States access to valid and trustworthy real-world evidence, for example on diseases, patient populations, and the use, safety and effectiveness of medicines, including vaccines, throughout the lifecycle of a medicinal product. The DARWIN-EU Coordination Center has been selected in 2022 and will be hosted by the Erasmus Medical Center in Rotterdam (the Netherlands). DARWIN-EU will also act as a pathfinder for the European Health Data Space (EHDS) and will ultimately connect to the EHDS services, enabling the use of the EHDS in the context of medicines regulation in Europe. The first DARWIN-EU pilot studies have been delivered in 2022. EMA will oversee the Coordination Centre, connect it to the work of the EMA medicines committees and monitor its performance. There are three main areas of EMA committees decision-making for which Real World Evidence from DARWIN-EU can be requested: 1) Support the planning and validity of applicant studies (e.g. design and feasibility of planned studies, representativeness and validity of completed studies), 2) Understand clinical context (e.g. disease epidemiology, clinical management and drug utilization, 3)

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	Investigate associations and impact (e.g. effectiveness and safety studies, impact of regulatory actions).
Category	Availability of data
Type of BB	Regulatory
Geographical scope	Europe
Availability	The first studies by DARWIN-EU started from 2022. These studies will be initiated based on questions from the regulatory authorities, such as EMA (e.g. EMA Scientific Committees) and the national European competent authorities. It is expected that in the long-term also questions coming from academia will be addressed, but no timelines are yet available.
Scope of use	Currently DARWIN-EU only focuses on questions from regulators.
Stakeholders involved	Regulators including EMA and national competent authorities, Erasmus University
Enablers/ Requirements	Be part of the DARWIN-EU network
Output	Real World Data that can assist in regulatory decision making. Below a few examples are provided: • to inform recruitment in pre and post authorisation studies: - number of incident and/or prevalent patients per year (for diseases and/or drug), geographical variation of incident and/or prevalent patients. • To evaluate external validity - measure the representativeness of the clinical trial population (treatment and control arm) versus the real-world target population, e.g., similar age distribution, severity of underlying illness.

ITEM	DESCRIPTION
	• To generate evidence on the actual clinical standards of care and compare in different populations - How are patients diagnosed and treated, treatment patterns.
Best time to apply and time window	Not applicable yet.
Expert tips	Not applicable yet. The use of DARWIN-EU is currently limited to regulators.