Orphan Drug Development Guidebook

Building Block E127

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	European Patients' Academy on Therapeutic Innovation (EUPATI) Toolbox
References	**EUPATI Toolbox** https://toolbox.eupati.eu/ **EUPATI website** https://www.eupati.eu/
Description	EUPATI is a multi-stakeholder public-private partnership originally launched by the IMI-EUPATI project (2012-2017) and hosted by the European Patients' Forum (EPF) from 2017 to 2020. EUPATI is today established as an independent non-profit Foundation in the Netherlands. It is a successful programme that provides education and training to increase the capacity and capability of patients and patient representatives to understand and meaningfully contribute to medicines research and development (R&D), and to improve the availability of medical information for patients and other stakeholders. **TOOLBOX** The EUPATI Toolbox is organised in two main areas: Medicines R&D and Patient Engagement. It hosts a variety of materials related to Medicines R&D. This includes articles, presentations, videos, webinars, starter kits, factsheets, guidance documents, infographics, and glossary items. Aside from the main articles that are found on the Toolbox, the EUPATI guidance documents and Webinars are core products that EUPATI offers.

	if you are interested in a certain Medicines R&D subject area, the toolbox includes a selection of 12 key introductory topics to help you choose from: Basics of Medicine R&D, Clinical Development / Trials, Drug Discovery, Personalised Medicine, Regulatory Affairs, Benefit and Risk Assessment, HTA, Pharmaceutical Development, Safety of Medicines, Non-Clinical Studies, Pharmacoepidemiology, Types of Medicines. At the moment, the Toolbox contains information in 13 languages (nonetheless, the Toolbox is continuously expanding and will soon be available in more languages).
Category	Developmental Resources Building Block
Geographical scope	European Union, Japan
Availability	The EUPATI Toolbox is not specific to Rare Diseases but provides a broad range of useful information. The information though is very relevant to rare disease medicines development and highly useful and appreciated by the rare disease patients.
Scope of use	Patient Training and use the EUPATI toolbox for advocacy Research but also experience in patient advocacy and activism show that experts and patients, and their organisations are an important part of medicines development. They can only achieve effective and sustainable results in advocacy if their knowledge about their disease areas and political environment is sound and scientifically founded. It is essential that experts and patients can clearly see the processes and methods by which medicines are developed and brought to the market to understand where and how they can make a meaningful impact, and how they can influence the process to make sure that patients' interests and viewpoints are duly represented. Based on your goal, EUPATI can advise you on how to achieve it.
Stakeholders	EUPATI is a public-private partnership composed of a multi-stakeholder structure representing patient organisations, not-for-profit, pharmaceutical industry and academic institutions.
Enablers / Requirements	none

Output	Access to scientific resources bringing patients advocates and researchers together.
Best time to apply and time window	No time window – essential when you want to start exploring medicines research and development as well as patient engagement.
Expert tips	PROs: − Focus on Research & Development – online training and resources – translation in several language and national coverage.