Orphan Drug Development Guidebook

Building Block E126

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	EURORDIS' Community Advisory Boards (CABs)
References	https://www.eurordis.org/get-involved/eurordis-community-advisory-board-programme/
Description	Patient Community Advisory Boards (CABs) are groups established and operated by patient advocates. They facilitate discussions, in a neutral setting, on the latest developments and challenges related to medical research and procedures in a specific disease area with the company or body conducting the research. For example, by being involved before a clinical study starts, patients help ensure that clinical studies are designed to take into account their real needs, resulting in higher quality research. CABs, with anywhere from seven to twenty patient advocate members, are involved in scientific as well as policy-related issues (ie, access). They provide expert advice to all stakeholders involved in the research, development and service provision of medical treatments.The CAB and the Companies are called to operate under confidentiality and strict but productive format. The result is a high-quality written advice that can be used as a guidance for the R&D but also as an unbiased advice of patient preferences in dealing with regulatory and access hurdlers. Key points are: – Code of Conduct, MoU and Confidentiality Agreement

	– Structured communication channels via the CAB's Secretariat of Chair that prohibits individual communications outside the CAB setup and provides the extra credibility to the advice given – High quality written advice from the patient community For a company, CABs represents an unbiased way to engage with the disease community knowledge, experience and its experts in a safe harbor setting and under CA. The company can use the produced advice report for R&D but also regulatory and HTA. For the patients, it is a way to provide advice to developers and researcher throughout the medicine's development cycle from pre-clinical to post marketing. It provides the opportunity to provide their unbiased opinions and preferences in a safe and patient-controlled environment. CABs were created in the 90s for HIV/AIDS, hepatitis and breast cancer. Some rare disease European federations, with or without EURORDIS support, have already gained experience with CAB-like initiatives.
Category	Developmental Resources Building Block
Geographical scope	European Union
Availability	Sponsors developing medicines for rare and non-rare diseases.
Scope of use	On a wide variety of topics that their members are the experts on: patient outreach, education on research, clinical studies and their design, criteria for participation, informed consent forms and processes, compassionate use programmes, retention of participants, and reporting on results. Input is also possible on disease-specific topics such as clinical endpoints and how they are measured, meaningful patient relevant outcomes (PROMs), disease registries and their common elements or factors that are meaningful to patients when measuring health and social outcomes.
Stakeholders	• Patient communities with their experts

	• All stakeholders that are involved in research, medicine development or policy making
Enablers / Requirements	• Educated patient communities • Proactive developers
Output	The output is higher quality research and faster access by imbedding patient as partners in the full cycle of development.
Best time to apply and time window	The tool has its use during the full cycle of medicines development from Preclinical to Post Marketing.
Expert tips	Contact Rob.Camp@eupati-es.org PROs: – CABs have been proven successful since more than 20 years within the HIV community – Trainings can be embedded into the CABs, Horizon scanning approaches, Identification of Unmet Medical Needs, etc CONs: – A CAB may not exist for all conditions