

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/content/eurordis-community-advisory-board-cab-programme
Description	<p>Patient Community Advisory Boards are groups established and operated by patient advocates. They facilitate discussions in a safe harbor setting and under strict rules, an MOU and CA on the latest developments and challenges related to medical research and procedures in a disease area with the company or body conducting the research. The CAB can cover from preclinical to post marketing topics. The CAB depending on the areas of discussion can invite scientific expert to serve as advisors.</p> <p>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 hurdles.</p> <p>Key points are:</p> <ul style="list-style-type: none"> – Strict Code of Conduct, MoU and CA – 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

	<p>– High quality written advice from the patient community</p> <p>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.</p> <p>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.</p> <p>For both it is the optimal alternative of a Sponsor driven CAB that is perceived biased by nature, lacks any representation and its results have no added value in the regulatory environment.</p> <p>When the independent patient CAB are organized by the Patient Organizations and their patient experts, the Industry one’s are no longer support and become obsolete.</p>
Category	Developmental Resources Building Block
Geographical scope	European Union
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	<p>CABs offer their expertise and advice to sponsors of clinical research. For example, by being involved before a clinical study starts, patients help ensure that clinical studies and their endpoints are relevant and meaningful. They consult the developers how to design CTs that take into account their real needs, managing the burden of participation and retention but also which are the clinically relevant outcomes. This is resulting in higher quality research and faster access.</p> <p>The experience has shown that once a CAB is functional Industry gets involved early and in a continues way during the development indications the high added value of the Patient driven CAB.</p>
Stakeholders	<ul style="list-style-type: none"> • Patient communities with their experts • All stakeholders that are involved in research, medicine development or policy making

Enablers / Require ments	<ul style="list-style-type: none"> • 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	<p>Contact rob.camp@eurordis.org</p> <p>PROs:</p> <ul style="list-style-type: none"> – 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 <p>CONs:</p> <ul style="list-style-type: none"> – A CAB may not exist for all conditions