

## Orphan Drug Development Guidebook

### Building Block I432

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	Horizon Scanning: Landscape analysis/ Stakeholder identification and engagement
References	<a href="https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4234281/">https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4234281/</a>
Description	Analysis performed by the drug developer and by Health Technology Assessment (HTA) bodies to assess: 1. the future real need for the therapy for the product at the time of market entry; 2. the potential future place in therapy for the product; 3. the current and future competitive landscape of an early-stage drug to determine the possibilities of market entry and the viability of possible differentiation strategies to compete in the market; 4. the best technology suitable to fulfil patient's need at the beginning of development.
Category	Development Resources Building Block
Geographical scope	International
Availability	This tool is developed for drug developers and for HTA bodies. A horizon scanning can be done for any drug that will be developed, not limited to an orphan medicinal product.
Scope of use	To project the clinical and market situation at the time of product launch.  To refine the target product profile and development plan, using this BB as a decision support tool for go-no-go decision making in drug development.

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	To allow Health Technology Assessment bodies prioritize new technologies and pharmaceuticals, based on the expected health benefit or financial impact, inappropriate use, and variation of use across the country and effect on other health-related policies, such as reduction in health inequalities.
Stakeholders	Drug developers, HTA bodies
Enablers/ Requirements	There are different tools available that could assist in horizon scanning, each with a different focus.
Output	Document
Best time to apply and time window	<p>Best to access early on in development to get full view of research and activities in the disease-specific space.</p> <p>To be produced at the start, periodically repeated throughout the development process.</p>
Expert tips	<p>Best developed at the very start of drug development.</p> <p><b>PROs:</b></p> <p>Benefits could be:</p> <ul style="list-style-type: none"> <li>• Understanding the therapeutic solution landscape, now and at the time of approval</li> <li>• Align the development of the product with the future potential place in therapy and existing therapeutic trends</li> <li>• To avoid investing resources in potentially useless or out of date targets/ drugs</li> <li>• To understand existing potential competitors or collaborators</li> <li>• Targeting the right group of patients considering the therapeutic toolkit at the time of approval</li> </ul> <p><b>CONs:</b></p> <p>Given the complexity of the therapeutic landscape in certain diseases, and drug development attrition rate, results of landscape</p>

ITEM	DESCRIPTION
	analysis should always be handled with care in informing drug development decisions to avoid errors in execution.