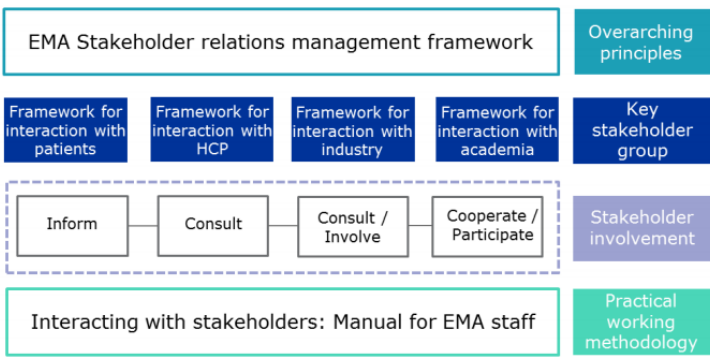


## Orphan Drug Development Guidebook

### Building Block E116

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	EMA framework for stakeholder's engagement – patients, academia, healthcare professionals
References	<a href="https://www.ema.europa.eu/en/documents/presentation/presentation-module-2-engagement-stakeholders_en.pdf">t https://www.ema.europa.eu/en/documents/presentation/presentation-module-2-engagement-stakeholders_en.pdf</a> <a href="http://www.ema.europa.eu/docs/en_GB/document_library/Other/2016/06/WC500208987.pdf">http://www.ema.europa.eu/docs/en_GB/document_library/Other/2016/06/WC500208987.pdf</a>
Description	<p>The Agency has been interacting with its stakeholders since its inception. These stakeholder relations have evolved over time and the type and degree of interaction is varied depending on the stakeholder groups and fields of Agency activity.</p> <p>Stakeholder interaction must be based on the fundamental principles:</p> <ul style="list-style-type: none"> <li>• Transparency</li> <li>• Independence and integrity</li> <li>• Accountability</li> <li>• Appropriate interaction</li> <li>• Broad representation</li> <li>• Effective communication</li> <li>• Continuous improvement</li> </ul>

	 <p>Together, these building blocks ensure a <b>consistent approach to stakeholder relations management</b> across a variety of stakeholder groups and interaction types.</p> <p>Continues interaction in and organised way is supporting high-quality and expedite drug development. The rare disease patients are imbedded in all EMA activities from EMA to committees to Scientific Advice (SA) and Protocol Assistance to oral explanations in CHMP. The same applies to the involvement of the rest of the stakeholders like Academia, Health Care Professionals (HCPs). This interaction promotes especially knowledge exchange and understanding of rare diseases.</p>
Category	Regulatory Building Block
Geographical scope	European Union
Availability	Organizations, associations and parties interacting with the European Medicines Agency (the Agency or EMA hereafter), which have an interest in or are influenced by the work of the EMA and its partners regarding rare and non-rare diseases.
Scope of use	<p>By applying the following principles, the Agency aims to structure stakeholder relations and better support its strategic priorities:</p> <ul style="list-style-type: none"> <li>• Promote appropriate engagement and dialogue on topics concerning medicines for human and veterinary use;</li> <li>• Improve communication to manage expectations and provide efficient, targeted and timely information, in a proactive manner;</li> <li>• Enhance stakeholders' understanding of the EU medicines Regulatory network and the role of regulators and enrich EMA's understanding of issues that are pertinent from the stakeholders' perspective throughout the lifecycle of a medicinal product;</li> </ul>

	<ul style="list-style-type: none"> <li>• Increase transparency on how EMA engages with stakeholders.</li> </ul>
Stakeholders	<ul style="list-style-type: none"> <li>• EMA</li> <li>• Patients and consumers' organizations</li> <li>• Healthcare professionals' organizations</li> <li>• Scientific and academic societies</li> <li>• Representative associations of human or veterinary pharmaceutical industry</li> </ul>
Enablers/ Requirements	<p>For all EMA interactions the general norm is "Come early and come often".</p> <p>Four levels of stakeholders involvement have been identified:</p> <ul style="list-style-type: none"> <li>• Inform (e.g. announcement of review of policy or guidance; information days);</li> <li>• Consult (written – e.g. public consultation on policies or guidance, surveys);</li> <li>• Consult and involve (direct interactions – e.g. stakeholder meetings, workshops, stakeholder conferences, public hearings);</li> <li>• Cooperate / participate (direct interactions - e.g. technical expert groups (Telematics, ENCePP, focus groups, technical expert groups, as appropriate).</li> </ul>
Output	Open, proactive and meaningful dialogue between all stakeholders and the EMA, for clear and transparent information.
Best time to apply and time window	Throughout the process of development.
Expert tips	<p>PROs:</p> <ul style="list-style-type: none"> <li>– Early interaction and knowledge exchange</li> </ul>