Orphan Drug Development Guidebook

Building Block E105

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 Medicines Agency's Scientific Advisory Groups (SAGs)
References	https://www.ema.europa.eu/en/committees/working-parties-domains/chmp-working-parties-other-groups Operational expert groups: Scientific Advisory Group on Cardiovascular Issues: https://www.ema.europa.eu/en/committees/working-parties-domains/chmp/scientific-advisory-group-cardiovascular-issues Scientific Advisory Group on Infectious Diseases: https://www.ema.europa.eu/en/committees/working-parties-other-groups/chmp/scientific-advisory-group-infectious-diseases Scientific Advisory Group on Neurology: https://www.ema.europa.eu/en/committees/working-parties-other-groups/chmp/scientific-advisory-group-neurology Scientific Advisory Group on Vaccines: https://www.ema.europa.eu/en/committees/working-parties-other-groups/chmp/scientific-advisory-group-vaccines Inter-Committee Scientific Advisory Group on Oncology: https://www.ema.europa.eu/en/committees/working-parties-other-groups/chmp/inter-committee-scientific-advisory-group-oncology
Description	SAGs can be convened by the Committee for Medicinal Products for Human Use (CHMP) to provide independent scientific expertise and advice relevant to the evaluation of medicines, when: • Expected major public health interest (e.g.: first-in-class) • Substantial disagreement between rapporteurs on clinical aspects • Controversial issues (e.g., high impact on health care professionals, the public and other stakeholders) • Complex technical aspects, rare diseases • Risk minimization measures affecting the clinical practice

	• Advice is needed on the design and feasibility of a clinical trial • Major post-authorization safety issues The SAGs deliver opinions, on a consultative basis, to specific questions raised by the CHMP. The CHMP decides on the duration of the SAG activity. Costs are included as part of the MAA process. See: https://www.ema.europa.eu/en/documents/other/procedural-advice-committee-medicinal-products-human-use-need-convene-scientific-advisory-group-ad_en.pdf -
Category	Regulatory Building Block
Geographical scope	European Union
Availability	The SAG activities are for rare and non-rare diseases.
Scope of use	The CHMP may establish scientific advisory groups to provide advice in connection with the evaluation of specific types of medicines or treatments at the time of Marketing Authorisation Application. They consist of European experts selected according to the particular expertise required on the basis of nominations from the CHMP or the Agency. Patient experts might be invited to SAGs by the EMA. A debriefing meeting with the company will occur after the end of the SAG meeting. A company/applicant may request that the Committee consult a SAG in connection with the re-examination of its opinion.
Stakeholders	• The CHMP and European independent experts. • Patient representatives can also be appointed to attend the SAG meeting. • The company/applicant or a third party may be invited to provide an oral explanation in front of the SAG.
Enablers/ Requirements	The CHMP considers gathering experts for a SAG meeting, whenever there are diverging opinions or complex clinical issues that are uncovered during the review process for a marketing authorisation. The SAG reports back to the CHMP on specific issues raised.

Output	SAG reports back to the CHMP on specific issues raised (i.e. clarification of the elements of the dossiers in view of a decision by CHMP)
Best time to apply and time window	CHMP may convene a SAG meeting in relation to a MAA application. SAGs can be convened also in the post-marketing phase.
Expert tips	Advice should be considered in the context of the MAA procedure. PROs: Focused discussion with experts of a particular medical field on a specific dossier or clinical or drug development issue. CONs: A company may only request a SAG in connection with the re-examination of its opinion.