Orphan Drug Development Guidebook

Building Block E104

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	National Member State Scientific Advice (NMS-SA)
References	Each Member State that offers scientific advice will have details on their own specific web site. The details vary between Member States. Examples include: https://www.gov.uk/guidance/medicines-get-scientific-advice-from-mhra http://www.bfarm.de/SharedDocs/Downloads/EN/Service/AdviceProcedures/Guidance_for_Applicants_ScientificAdvice.pdf?__blob=publicationFile&v=3 https://english.cbg-meb.nl/documents/leaflets/2017/01/01/scientific-advice https://mapbiopharma.com/italy/reimbursement/scientific-advice/
Description	Procedures set up by many (but not all) National Member State to offer scientific advice to developers of new medicines. The procedures can be similar but often less formal than for CHMP scientific advice/protocol assistance at the EMA; they may include written advice and/or face-to-face meetings. The procedures are not limited only to orphan product development and may vary between Member States. Often meetings can be arranged within 6 – 8 weeks of a request; sometimes this may be 6 – months, depending on the Agency, types of issues to discuss, competing workload, etc. Agency fees vary: some offer this advice for free, others charge a fee.

Category	Regulatory Building Block
Geographical scope	European Union
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	Many developers request advice from Member States before approaching CHMP for advice – it can be a way to discuss major issues regarding the development of the prodruct before submitting a much more extensive request to CHMP for advice. Used when there is a lack of clear regulatory guidance or precedent for how to develop a particular medicine.
Stakeholders	National Member State regulatory agencies (possibly including external experts) Drug developers (possibly including external experts / KOLs)
Enablers / Requirements	No pre-requirements.
Output	The output may vary by Member State. All will give advice and answers to specific questions: some give oral advice (at a meeting); some write formal minutes/advice letters; some comment on sponsor's own minutes.
Best time to apply and time window	The tool has its use any time from the starting of the clinical development up to before Marketing Authorization Application submission. In most cases, the best time is when you enter clinical phase.
Expert tips	Do not expect a "pre-assessment" of a pending MAA. Ensure advice sought relates to plans for future development, not simply review of already obtained study results. Advice given is usually not binding on the regulator.

	PROs: • Usually a quicker, cheaper, and less formal procedure than obtaining scientific advice from CHMP. CONs: • All orphan products will eventually be appraised by CHMP and these national advice meetings may not always represent a Europe-wide opinion.