Orphan Drug Development Guidebook

Building Block E107

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	Accelerated Assessment (EMA-AA)
References	https://www.ema.europa.eu/en/human-regulatory/marketing-authorisation/accelerated-assessment
Description	The accelerated assessment allows a faster assessment process for the marketing authorization application under the Centralised Procedure: from 210 days (standard procedure) to 150 days approximately (excluding clock stops when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP). Days have to be considered as *procedural days* rather than *calendar days*. The time frame is divided in 3 phases: 90+30+30 days. ("The applicants will be allowed to have one month clock-stop by default for preparation of responses to Day 90 List of Questions and no clock stop by default after Day 120 List of Outstanding Issues".) In case of advanced therapy medicinal products (medicines based on genes, cells or tissue engineering), the 150 days is adapted to 120+30 days of assessment.
Category	Regulatory Building Block
Geographical scope	European Union

Availability	The procedure is available for any Applicant (regardless of their legal entity) developing medicines for rare and non-rare diseases, providing that the applicant can justify their claim that the medicinal product is expected to be of major public health interest, particularly from the point of view of therapeutic innovation.
Scope of use	Used by drug developers and regulators to provide accelerated patient access to new medicines "of major interest for public health and with therapeutic innovation".
Stakeholders	Any applicant (regardless of their legal entity)
Enablers/ Requirements	Applicants requesting an accelerated assessment procedure should justify that the medicinal product is expected to be of major public health interest.
Output	Based on the request, the justifications presented, and the recommendations of the Rapporteurs, the CHMP will formulate a decision. Such a decision will be taken without prejudice to the CHMP opinion (positive or negative) on the granting of a marketing authorisation.
Best time to apply and time window	- EMA strongly recommends that applicants request a pre-submission meeting six to seven months before submission to prepare for evaluation under accelerated assessment. - Any request for accelerated assessment should then be made at least two to three months before submitting the marketing-authorisation application.
Expert tips	- Evidence requirements for applications to be assessed under accelerated assessment are the same as for other applications - Under the PRIME scheme, it is possible for applicants to receive confirmation during the clinical development phase that their medicine might potentially be eligible for accelerated assessment - The request should be presented as a short but comprehensive document (ideal length 5-10 pages) PROs: - Shorter centralized MAA review timelines equals to earlier patient access to medicines of major interest for public health and with therapeutic innovation

	CONs: - Tighter timelines to address CHMP response: 1 month vs. 3 months. - If the responses are not provided within 1 month, then the MAA review exits the accelerated review path and continues under the standard review timing for Centralised MAA