Orphan Drug Development Guidebook

Building Block U206

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	FDA Expedited Program for serious conditions - Priority Review Designation (FDA-PR)
References	https://www.fda.gov/ForPatients/Approvals/Fast/ucm405405.htm https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM358301.pdf
Description	A PR results in an abbreviated PDUFA goal date for a Biologics License Application (BLA) or a New Drug Application (NDA), from 10 months (under standard procedure) to 6 months. Like other expedited programs, the qualifying criteria do not require that the therapeutic in development target a rare disease. Submitting a request for PR has no cost. PDUFA filing fees, however, should be assessed contemporaneously.
Category	Regulatory Building Block
Geographical scope	United States of America

Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	A Priority Review designation will direct overall attention and resources to the evaluation of applications for drugs that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications.
Stakeholders	• NDA and BLA Applicants • FDA
Enablers / Requirements	• Treat a serious condition (a disease or condition associated with morbidity that has substantial impact on day-to-day functioning). • Demonstrate the potential to be a significant improvement in safety or effectiveness as, for example: 1. evidence of increased effectiveness in treatment, prevention, or diagnosis of condition; 2. elimination or substantial reduction of a treatment-limiting drug reaction; 3. documented enhancement of patient compliance that is expected to lead to an improvement in serious outcomes; or 4. evidence of safety and effectiveness in a new subpopulation.
Output	Designation enabling Marketing application review process within 6 months of receipt of marketing application.
Best time to apply and time window	With original BLA, NDA, or efficacy supplement (FDA responds within 60 calendar days of receipt of these applications).
Expert tips	When Applicants receive an expedited drug development designation, they should be prepared to propose a commercial manufacturing program that will ensure availability of quality product at the time of approval. The proposal should consider estimated market demand and the commercial manufacturing development plan. The proposal should also consider manufacturing facilities and a lifecycle approach to process validation.

	Additionally, the proposal should include a timeline for development of the manufacturing capabilities with goals aligned with the clinical development program. Not to be confused with US FDA Priority Review Voucher (PRV) programs. Applicants should consider that the resources required to expedite review by FDA should be mirrored by the Applicant. Net impact being that an Applicant will have less time to respond to Information Requests, prepare for Advisory Committee, if applicable, etc. in the case of a Priority Review. PROs: – Shorter clock for review of marketing application (6 months compared with the 10-month standard review) – FDA decides on the review designation for every application CONs: – A 60 day filing review period, in which FDA makes a determination that the application is sufficiently complete to permit a substantive review, for some NDA/BLA submissions is applied to both Standard and Priority Review. – Note that a Priority Review in no way lowers the standard of evidence and quality of data required by FDA. It is possible that a reduced timeframe to respond to FDA reviewer requests can lead to Applicant mediated delays or even a complete response.