IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

Orphan Drug Development Guidebook

Building Block U209

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	Rare Pediatric Priority Review Voucher
References	https://www.fda.gov/forindustry/developingproductsforrarediseasesconditions/rarepediatricdiseasepriorityvoucherprogram/default.htm https://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM423325.pdf
Description	This program grants a voucher for priority review from the US Food and Drug Administration (FDA), which aim to render a decision within 6 months (in contrast to 10 for a standard review). The developed drug for which the voucher is awarded must be intended for a rare disease or condition and that primarily affect individuals from 0 to 18 years. The voucher can be utilized for any other drug development program. The FDA has to render a decision within 6 months after receipt of the application and will establish the fee amount before the beginning of each fiscal year. Applications may be eligible for exemptions from some fees if they have received orphan-drug designation.
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Applicants developing medicines for rare pediatric diseases.

Scope of use	The Voucher is used for a different (generally non-orphan) drug program. The granting of the Voucher is a financial incentive for the orphan pediatric drug program to which it is awarded.
Stakeholders	• Developers • The US Food and Drug Administration (FDA) • The Office of Orphan Products Development (OOPD) • The Center for Drug Evaluation and research (CDER) • The Center for Biologics Evaluation and research (CBER)
Enablers / Requirements	• The drug must be intended for a rare disease or condition • The drug must be intended for a disease or a condition that "primarily affects individuals from 0 to 18 years of age" • The developed drug cannot contain any active ingredient (including any ester or salt of the active ingredient) of which has been approved in any other application • FDA review of the request for a Rare Pediatric Priority Review Voucher can be expedited if the pediatric orphan drug is pre-designated as a "Rare Pediatric Disease" by FDA
Output	The Voucher, when utilized for a different drug development program, expedites FDA review: 6 months, compared to 10 months for a standard application.
Best time to apply and time window	At the time of marketing authorisation submission.
Expert tips	NB: It is not necessary to be granted a "Rare Pediatric Disease" to request a rare pediatric priority review voucher, although previous application and granting of "Rare Pediatric Disease" status may expedite FDA review for a Voucher. PROs: • Provides financial incentives for developing pediatric orphan drugs.

	CONs: • The financial incentives to the orphan drug program are realized once the Voucher is sold to a different program. As the Voucher is not awarded until the marketing approval of the pediatric orphan drug, the incentive appears relatively late in the drug development pathway for the orphan drug to which it is granted. • There have been examples of 'gaming the system' where a drug approved in EU for common disease, but not approved for any indication by FDA, was taken forward selectively in a rare pediatric indication in the US, in part, due to awarding of a Voucher.