Orphan Drug Development Guidebook

Building Block U208

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 Disease Designation
References	https://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM423325.pdf
Description	The FDA grants rare pediatric disease designation for diseases with serious or life-threatening conditions that affect people from birth to 18 years of age, that affect fewer than 200,000 people in the U.S. The purpose of this designation is to enable later application for a Rare Pediatric Disease Priority Voucher at the time of drug approval (NDA/BLA). Such a Voucher can be redeemed to grant priority review of a subsequent marketing application for a different product. Vouchers are thus transferred for use in some other program to achieve "priority review" status, thus expediting the regulatory review of this other drug program. Such vouchers are typically sold by the Sponsor of the Rare Pediatric Disease drug program, to apply to some unrelated drug development program (typically by a different Sponsor). https://www.fda.gov/forindustry/developingproductsforrarediseasesconditions/rarepediatricdiseasepriorityvoucherprogram/default.htm
Category	Regulatory Building Block
Geographical scope	United States of America

Availability	Applicants developing medicines for Rare Pediatric Diseases.
Scope of use	The Rare Pediatric Disease Designation is based on voluntary requests and is not required for submitting a Rare Pediatric Priority Review voucher. While such designation is not required to receive a voucher, requesting this in advance will expedite a sponsor's future request for a priority review voucher. On the other hand, if sponsors choose to request a rare pediatric disease designation, they should also submit requests for Orphan Drug Designation or a Fast Track Designation. To expedite future applications for a Rare Pediatric Disease Priority Voucher.
Stakeholders	• Developers • The US Food and Drug Administration (FDA) • The Office of Orphan Products Development ((Building Block 42) • 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
Output	The final product is a designation.
Best time to apply and time window	Before the FDA has filed for the New Drug Application (NDA) and Biologics License Application (BLA) for the drug.
Expert tips	NB: It is <u>not</u> necessary to be granted a “Rare Pediatric Disease” designation to request a rare pediatric priority review voucher (Building Block U208). However, requesting this designation in advance will expedite a sponsor's future request for a priority review voucher.

	PROs: • Designation can expedite future granting of a Rare Pediatric Disease Priority Voucher upon marketing authorization. • The Voucher can then be sold to other drug programs, providing Priority Review. • A relatively straightforward and simple process, that receives relatively rapid review. CONs: • The Designation does not guarantee granting of a future Voucher. • The Designation is not necessary for granting of a future Voucher.