Orphan Drug Development Guidebook

Building Block U210

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	Neglected and Tropical Diseases Priority Review Voucher
References	https://www.fda.gov/downloads/Drugs/Guidances/UCM080599.pdf
Description	This program grants a priority review voucher (PRV) from the US Food and Drug Administration (FDA) to sponsors of certain tropical disease product applications (see list below). The PRV can be used to obtain a priority review (PR) for a subsequent human drug application after the date of approval of the tropical disease drug product. FDA aims to review and act upon a PR NDA or BLA submission within 6 months of submission, rather than within 10 months for a standard review. A PRV can be used to obtain a PR for a subsequent application that would not otherwise qualify for a PR. After approval to the tropical disease product, the sponsor may redeem the voucher to obtain a PR for a subsequent human drug application (NDA or BLA) or may transfer the PRV to another party (e.g., may sell the voucher to another party). PRVs have monetary value that may benefit the seller of the PRV to encourage neglected and tropical disease product development, most of which are rare diseases in the US. There is no cost to request a PRV. The NDA/BLA application fee will still apply when using a priority voucher and requesting a PR, which ranges from $2-3 million. In case of Orphan drug designation, fees waiver according to section 736 of the FD&C For those sponsors who receive a PRV for a qualifying tropical disease, the FDA “anticipate that some tropical disease products may qualify for designation as orphan drugs because the tropical diseases for which these drugs are intended to prevent or treat may affect fewer than 200,000 persons in the United States (see section 526 of the FD&C Act). If a

	human drug application for a prescription drug product has been designated as a drug product for a rare disease or condition, the application is not subject to an application user fee, unless the application includes an indication other than for a rare disease or condition."
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Applicants developing medicines for a tropical disease. According to the FDA, a tropical disease is any of the following: • Blinding trachoma • Buruli Ulcer • Chagas • Chikungunya virus disease (added August 2018) • Cholera • Cryptococcal meningitis (added August 2018) • Cueva virus Diseases • Dengue/Dengue haemorrhagic fever • Dracunculiasis (guinea-worm disease) • Ebola virus Diseases • Fascioliasis • Filovirus Diseases • Human African trypanosomiasis • Lassa fever (added August 2018) • Leishmaniasis • Leprosy • Lymphatic filariasis • Malaria • Marburg virus Diseases • Neurocysticircosis • Onchocerciasis • Rabies (added August 2018) • Schistosomiasis • Soil transmitted helminthiasis • Tuberculosis • Yaws • Zika Virus Disease

	• Any other infectious disease for which there is no significant market in developed nations and that disproportionately affects poor and marginalized populations, designated by order of the Secretary
Scope of use	The PRV may be redeemed for any NDA or BLA application submitted to the US FDA. PRVs may also be transferred (sold) to another party for future redemption. For an application to qualify, it must be for a human drug, no active ingredient (including any ester or salt of the active ingredient) of which has been approved in any other application.
Stakeholders	• Developers • The US Food and Drug Administration (FDA) • The Center for Drug Evaluation and Research (CDER) • The Center for Biologics Evaluation and Research (CBER)
Enablers / Requirements	• The drug must be intended for prevention or treatment of a tropical neglected disease. • The drug must otherwise be eligible for a priority voucher • The drug must not contain any active ingredient that has been approved in any other application
Output	The FDA wards priority review vouchers to sponsors of certain tropical disease product Food, Drug, and Cosmetic Act (the FD&C Act) (21 U.S.C. 360n) applications that meet the criteria specified in the FDA guidance.
Best time to apply and time window	No application is required, but sponsors can request consideration for a PRV at the time of marketing application submission. FDA will award PRVs at the time of approval for qualifying applications.
Expert tips	"It is possible that a drug product meeting the requirements of a tropical disease also may qualify for designation as an orphan drug. If designated as an orphan drug, such a drug product may be eligible for orphan drug marketing exclusivity and tax credits for qualified clinical testing as well as fee exemptions. For information regarding these orphan drug

	incentives, potential sponsors should contact the Office of Orphan Products Development (OOPD). For information regarding user fee exemptions, potential sponsors should contact the User Fee staff in the Center for Drug Evaluation and Research's (CDER's) Office of Management." PROs: • A PRV is expected to result in monetary advantages for the recipient, either through a faster review and action upon a future application for the drug sponsor, or through sale of the voucher to another party. • The PRV program is intended to provide an incentive to sponsors for the development and marketing of tropical disease products.