Orphan Drug Development Guidebook

Building Block U201

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	USA Orphan Drug Designation
References	FDA Orphan Drug Designation Program Website: https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/designating-orphan-product-drugs-and-biological-products
Description	The Orphan Drug Act (ODA) provides for granting special financial incentives to a sponsor developing a drug or biological product ("drug") for use in a rare disease or condition upon request of that sponsor. This status is referred to as orphan designation (or sometimes "orphan status"). A rare disease or condition is defined as any disease or condition which: – Affects less than 200,000 persons in the US, or – Affects more than 200,000 in the US and for which there is no reasonable expectation that the cost of research and development of the drug for the indicationcan be recovered from sales of the drug in the US. Sponsors seeking orphan drug designation for a drug must submit a request for designation to FDA. Following receipt of the request for orphan drug designation by theagency, the request is typically reviewed in a cycle of approximately 90 days: • Will either receive: – Designation Letter OR – Deficiency Letter • Once designated, sponsor is required to submit annual reports until drug is approved for the designated use.

ITEM	DESCRIPTION
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Sponsors developing medicines for rare diseases.
Scope of use	The goal of this building block, along with other tools of the FDA Office of Orphan Products Development (OOPD), is to advance the development of products (drugs, biologics, devices, or medical foods) that demonstrate promise for rare diseases and conditions. A sponsor may seek orphan drug designation for a specified rare disease or condition of a previously unapproved drug, or of a new use for an already marketed drug.
Stakeholders	• Sponsor • FDA, OOPD • FDA review center, Center for Biologics Evaluation and Research (CBER) • FDA review center, Center for Drug Evaluation and Research (CDER)
Enablers/ Requirements	The disease or condition for which the drug is intended affects fewer than 200,000 people in the United States or, if the drug is a vaccine, diagnostic drug, or preventive drug, the persons to whom the drug will be administered in the United States are fewer than 200,000 per year. For a drug intended for diseases or conditions affecting 200,000 or more people, or for a vaccine, diagnostic drug, or preventive drug to be administered to 200,000 or more persons per year in the United States, there is no reasonable expectation that costs of research and development of the drug for the indication can be recovered by sales of the drug in the United States. The scientific rationale portion of the orphan drug designation application must include enough information to establish a medically plausible basis for expecting the drug to be effective in the rare disease.

ITEM	DESCRIPTION
Output	Orphan Drug Designation
Best time to apply and time window	A sponsor may request orphan drug designation at any time in its drug development process prior to the time that the sponsor submits a marketing application for the drug for the same rare disease or condition.
Expert tips	A resource for sponsors in compiling the orphan drug request is the Recommended Tips for Creating an Orphan Drug Designation Application webinar, slides and script. Thisinformation as well as the orphan drug designation request form, frequently askedquestions about orphan drug designation, and other topics are available on the FDA's Officeof Orphan Products Development website: https://www.fda.gov/industry/medical-products-rare-diseases-and- conditions/designating-orphan-product-drugs-and-biological-products PROs: – Orphan designation provides incentives including: o Tax credits for qualified clinical trials o Exemption from user fees o Potential for seven years of market exclusivity after approval CONs: – There are no risks. There is no cost to submit an orphan drug designation request to FDA, and a negative decision does not otherwise undermine the development of the drug for its intended use.