Orphan Drug Development Guidebook

Building Block U215

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	Product specific – pre-approval advisory committee
References	https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/ucm079765.pdf https://www.fda.gov/downloads/regulatoryinformation/guidances/ucm125650.pdf
Description	Advisory committees (AC) provide independent advice and recommendations the US FDA on scientific and technical matters related to the development and evaluation of products regulated by the FDA. Through the AC system, FDA is able to secure independent professional expertise in accomplishing its mission and maintaining the public trust. Although the AC provides recommendations to FDA, final decisions are made by FDA. The US FDA convenes advisory committee meetings for a variety of different purposes, some of which include pre-approval meetings for marketing application submissions for NDAs, BLAs, and supplication supplements. Meetings will differ depending on the issues relevant to the application under review, but may include issues related to the product's efficacy, safety, manufacturing or characteristics, and labeling or intended use, among others. ACs are not specific to Rare Diseases, but are frequently convened for novel products (e.g., new classes of drugs or innovative therapies), for scientific questions (such as non-traditional trial designs or small clinical development programs), or to provide expert advice on difficult questions. These situations are not uncommon in rare diseases drug development and thus, ACs are likely to be convened for many rare disease products.

	ACs typically occur within the last 60-90 days of a product review cycle. ACs typically last 0.5-2 days, depending on scope of issues to be discussed.
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Advisory committee meetings may occur for any product and or not specific to rare diseases; however, novel classes of drugs, novel treatment approaches or first-in-disease drugs are frequently the subject advisory committee meetings, which have applicability to orphan drug development, review and approval.
Scope of use	The goal of this building block is to assist product developers in anticipating and preparing for an FDA advisory committee, should one be convened. ACs also generally have an Open Public Hearing portion at which any member of the public may apply to speak, including patients, caregivers and other interested parties in the rare disease community. Written comments may also be submitted to the AC by members of the public.
Stakeholders	• Product developers • FDA CDER, CBER • External experts who comprise the independent Advisory Committee
Enablers/ Requirements	Advisory Committee meetings are convened at the request of FDA, who may seek external expert input into issues related to products under-going review or oversight.
Output	There are several outputs for an AC: • Public posting of the meeting shall occur approximately 60 days in advance of an AC • Briefing documents prepared in advance of an AC are compiled by the drug sponsor and by the FDA review division with regulatory oversight for the product. Briefing packages are generally published on the FDA's website within 7 days of an AC meeting

	• For an open meeting, sponsor and FDA presentations will be made during the AC as well as discussions and voting on individual questions on the application before the committee. Closed meetings are not open to the public, and written materials related to a close meeting will not be made publicly available. • A meeting transcript, minutes, and all meeting materials will be published on the FDA website generally within 90 days after a meeting.
Best time to apply and time window	FDA staff convene a meeting and there is no application process. A sponsor may request that an AC be held, which typically would occur during product review of an NDA, BLA or supplement; however, an AC may be convened at any time should issues requiring independent advice arise (e.g., a safety concern in the post-marketing period).
Expert tips	Consult the FDA AC website for additional information: https://www.fda.gov/AdvisoryCommittees/default.htm Developers may also request assistance for preparing for a meeting from FDA AC and review division staff.