Orphan Drug Development Guidebook

Building Block U216

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	FDA Pediatrics Advisory Committee
References	https://www.fda.gov/advisorycommittees/committeesmeetingmaterials/pediatricadvisorycommittee/default.htm https://www.fda.gov/AdvisoryCommittees/CommitteesMeetingMaterials/PediatricAdvisoryCommittee/ucm116525.htm https://www.fda.gov/scienceresearch/specialtopics/pediatrictherapeuticsresearch/ucm123229.htm
Description	The Pediatric Advisory Committee (PAC) is a committee of authorities knowledgeable in pediatric research, pediatric subspecialties, statistics, and/or biomedical ethics who advise and make recommendations to the FDA regarding: 1) Pediatric research being conducted; 2) Identification of research priorities related to pediatric therapeutics and the need for additional treatments of specific pediatric diseases or conditions; 3) The ethics, design, and analysis of clinical trials related to pediatric therapeutics; 4) Pediatric labeling disputes per the Best Pharmaceuticals for Children Act (BPCA); 5) Pediatric labeling changes per BPCA; 6) Post-marketing adverse event reviews for drugs granted-pediatric exclusivity; 7) Any other pediatric issue or labeling dispute; 8) Research involving children as subjects;

	9) Any other matter involving pediatrics for which FDA has regulatory responsibility; 10) Aspects of Humanitarian Device Exemptions (HDEs) for certain HDE devices that are approved in pediatric patients. Most serious disorders involving children are rare, thus some of the issues brought before the PAC are likely to involve rare disease drug development. PAC meetings last 1-2 days and happen four times per year.
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	PAC meetings may occur for any product or issue relevant to pediatrics, and or not specific to rare diseases; however, it is likely a number of issues put to the PAC will have applicability to orphan drug development, review and approval.
Scope of use	Pediatric Advisor Committees are convened by the FDA in order to receive scientific or technical advice from an independent panel of experts relating to the review and oversight of products for and research conducted in pediatric patients. The goal of this building block is to assist product developers in anticipating and preparing for an FDA advisory committee, should one be convened, and to be aware of issues being brought before the committee. Public participation via requests to speak or written comments may be submitted to the PAC.
Stakeholders	• FDA Center of Drugs Evaluation and Research (CDER), Center for Biologics Evaluation and Research (CBER), Office of the Commission Office of Pediatrics, Office of Orphan Products Development • External experts who comprise the independent Advisory Committee • Members of the public
Enablers/ Requirements	The PAC is a standing committee that is convened by the FDA approximately 4 times per year.

Output	• Public notice for the meeting shall be posted on FDA's website approximately 60 days in advance of a PAC • Meeting materials including agendas, meeting rosters, briefing materials, presentations, meeting minutes and meeting transcript shall be posted prior to and within about 90 days after the meeting has occurred. https://www.fda.gov/advisory-committees/pediatric-advisory-committee/2021-meeting-materials-pediatric-advisory-committee
Best time to apply and time window	Typically occurs when matters of interest to pediatric drug review and oversight are needed.
Expert tips	Interested parties may also contact FDA staff at: • The Office of Pediatric Therapeutics: https://www.fda.gov/about-fda/office-clinical-policy-and-programs/office-pediatric-therapeutics • The Center of Drugs Evaluation and Research (CDER) Division of Pediatric and Maternal Health: https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/division-pediatric-and-maternal-health A dedicated updated page contains notices of advisory committee meetings https://www.fda.gov/advisory-committees/advisory-committee-calendar PROs: – Children are a special research population for whom additional oversight and independent advice are appropriate CONs: – Public airing of unsettled or controversial issues