Orphan Drug Development Guidebook

Building Block U212

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 Milestones meetings – Type B
References	https://www.accessdata.fda.gov/cder/sb-navigate/topic3/topic3/da_01_03_0110.htm https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM590547.pdf
Description	Type B meetings are also known as milestone meetings. Per the Draft Guidance for Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products, examples of type B meetings include: 1) Pre-investigational new drug application (pre-IND) meetings. 2) Pre-emergency use authorization meetings 3) Pre-new drug application (pre-NDA)/pre-biologics license application (pre-BLA) meetings 4) Post-action meetings requested 3 or more months after an FDA regulatory action other than an approval (such as issuance of a complete response letter). 5) Meetings regarding risk evaluation and mitigation strategies or post-marketing requirements that occur outside the context of the review of a marketing application. 6) Meetings held to discuss the overall development program for products granted breakthrough therapy designation status. Subsequent meetings for breakthrough

	therapy designated products will be considered either Type B or possibly Type A meetings if the meeting request meets the criteria for a Type A meeting. The default drug development program includes three milestones, or Type B meetings. The earliest is the Pre-IND Meeting, the second is the End of Phase 2 (EOP2) Meeting, and the third milestone is the Pre-NDA Meeting. Under the Prescription Drug User Fee Act (PDUFA), meeting management goals have been established to assist requesters who seek advice relating to the development and review of investigational new drugs and biologics, and drug or biological product marketing applications. Type B meetings apply to all drugs in development, including those intending to treat rare diseases. After requesting the meeting, FDA will confirm the scheduling within 21 days, and the meeting will take place no later than 60 days later. Official minutes of the meeting will be issued by FDA within 30 days of the meeting.
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Applicants who can be based in a public, non-profit, university, or private company developing medicines for rare and non-rare diseases.
Scope of use	Helps address specific regulatory concerns between a sponsor and the FDA at key milestone points in the development program. The Pre-IND meeting reviews the clinical development plans, the EOP2 meeting reviews the Phase 3 program plans, and the Pre-NDA meeting determines if the data package warrants submission of an NDA or BLA. Utilize a type B meeting to discuss the topics raised in description above at key milestones during development.
Stakeholders	• FDA • Drug developers
Enablers / Requirements	To submit a meeting request, per the reference above you must include “The proposed meeting format, […] The date the meeting background package will be sent by the requester. […] A brief statement of the purpose of the meeting. […] A list of the specific objectives or outcomes the requester expects from the meeting. […] A proposed agenda,

	including estimated times needed for discussion of each agenda item. […] A list of planned attendees from the requester's organization, including their names and titles. […] A list of requested FDA attendees and/or discipline representative(s)." Per the above reference, you should include "The application number (if previously assigned). […] The product name […] The chemical name, established name, and/or structure. […] The proposed regulatory pathway (e.g.; 505(b)(1), 505(b)(2). […] The proposed indication(s) or context of product development. […] The meeting type being requested […] Pediatric study plans, if applicable. […] Combination product information […] Suggested dates and times (e.g., morning or afternoon) for the meeting that are consistent with the appropriate scheduling time frame for the meeting type being requested […] A list of proposed questions, grouped by FDA discipline.
Output	Clarity about FDA requirements in order to be able to proceed successfully to the next step in the development process. Meeting minutes will be issued to the requester within 30 calendar days after the meeting.
Best time to apply and time window	Varies, based on issues raised / stage of development as outlined in description of types of Type B meetings above.
Expert tips	Contact FDA and seek input early. PROs: – Obtain clear input from the FDA on navigating the regulatory issues at various stages of therapeutic development. CONs: – Communications with FDA should not be limited to milestone, type B, meetings.