Orphan Drug Development Guidebook

Building Block U214

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	Pre-Investigational New Drug (IND) interactions
References	https://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm362746.htm https://www.fda.gov/downloads/Drugs/Guidances/UCM446695.pdf
Description	A pre-IND meeting is an FDA milestone meeting also known as Type B meeting (see BB U212). In these meetings the drug sponsor seeks FDA feedback about the quality, non-clinical and clinical development plan. The Pre-IND meetings are not mandatory. Current Federal law requires that a drug be the subject of an approved marketing application before it is transported or distributed across state lines. Because a sponsor will probably want to ship the investigational drug to clinical investigators in many states, it must seek an exemption from that legal requirement. The IND is the means through which the sponsor technically obtains this exemption from the Food and Drug Administration (FDA). A pre-IND meeting may be requested for issues related to data needed to support the rationale for testing a drug in humans; the design of nonclinical pharmacology and toxicology studies including design and potential uses of any proposed treatment in animal models; data requirements for an IND application; initial drug development plans, regulatory requirements for demonstrating safety and efficacy and other aspects of the development program. In terms of duration, it is scheduled within 60 days of written request.

Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	The Pre-Investigational New Drug Application (IND) Consultation Program fosters early communications between sponsors and the Center for Drug Evaluation and Research (CDER) new drug review divisions to provide guidance on the data necessary to warrant IND submission. All programs can be reviewed in a Pre-IND meeting, including those intending to treat rare diseases. The pre-IND feedback can help identify studies to support the initiation of clinical trials and can provide insight as to whether development can be eligible for an orphan drug designation.
Stakeholders	Any sponsor-investigator who is preparing and/or submitting complete IND applications to the CDER and the Center for Biologics Evaluation and Research (CBER) at the FDA. (A sponsor-investigator is an individual who both initiates and conducts an investigation, and under whose immediate direction the investigational drug is administered or dispensed. The term, as defined in FDA regulations, does not include any entity other than an individual.)
Enablers / Requirements	In order to benefit from pre-IND interactions with the CDER, sponsors must fill an IND application.
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	The pre-IND meeting has to be considered well ahead of the original IND submission date, so that there is enough time to generate and complete the original IND dossier requirements.

Expert tips	• Pre-IND meetings are not mandatory; however, they are highly recommended to minimize the risk of hold upon IND original submission. • The list of proposed questions is the most important element in the agenda. Each question should be precise. For each question, there should be a brief explanation of the context and purpose of the question. • You can ask to meet face-to-face, to have the meeting via teleconference or to receive written feedback. PROs: • Obtain clear input from the FDA on navigating the regulatory issues at various stages of therapeutic development. Particularly useful for small sponsors with limited drug development experience. CONs: • You can only have one Pre-IND meeting per application.