Orphan Drug Development Guidebook

Building Block U221

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 Patient-focused drug development program
References	FDA Patient-focused drug development: https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm Externally-led patient-focused drug development: https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm453856.htm
Description	The Patient-focused Drug Development (PFDD) program was initiated at FDA from 2012-2017 under Congressional Mandate (PDUFA V) to more systematically obtain the patient perspective on specific diseases and their treatments. PFDD meetings provide key stakeholders an opportunity to hear the patient's voice. From 2012-2017, 24 disease-area FDA-sponsored PFDD meetings were conducted for diverse disease areas, including common and rare diseases. In approx. 2017, the program was expanded to include Externally Led (EL)-PFDD meetings, where patient organizations undertook identifying and organizing patient-focused collaborations to generate public input on additional disease areas, using the process established through the PFDD model. EL-PFDD meetings are similar in intent to PFDDs, as opportunities for key stakeholders to hear the patient's voice and should target disease areas where there is an identified need for patient input on topics related to drug development. Some considerations in planning an EL-PFDD include: • An identified need to better understand the patient perspective for drug development purposes

	• Disease areas that are chronic, symptomatic, affect functioning and/or activities of daily living • For which aspects of the disease are not formally captured in clinical trials • For which there are currently no or very few therapies, or available therapies do not directly affect how a patient feels, functions or survives. • That have a severe impact on identifiable subpopulations (e.g., children, the elderly) The patient perspective is critical in helping FDA understand the context in which regulatory decisions are made for new drugs. While applicable to all diseases, this is especially relevant to rare diseases because rare diseases tend to be poorly understood, have little to no clinical trials or outcome assessment precedent, and the clinical manifestations of the rare disease are often highly heterogeneous and capturing the full range of patient experience is of substantial importance. Most meetings typically last 1 day, however the planning process could take about 1 year.
Category	Regulatory Building Block, Development Practices
Geographical scope	United States of America
Availability	EL-PFDDs may occur for any disease area, common or rare, and may be undertaken by key stakeholders. EL-PFDDs are highly relevant to rare diseases given the need to better understand the patient voice in disease progression, characteristics, and aspects of the disease of importance to patients.
Scope of use	The goal of this building block is to advance evaluation and development of products for rare diseases, especially disease natural history, potential outcome measures, and areas of clinical impact that are of particular importance to patients, through hearing the patient's voice and understanding the patient perspective in disease-specific areas. The Voice of the patient has tremendous value in rare diseases drug development in providing patient perspectives on aspects of the disease that are important to the patient. This information can contribute as a brief/partial cross-sectional natural history and provide information to identify outcome measures or areas for endpoint and clinical trial design development, as well as engaging the patient community in the drug development process for individual rare diseases.

Stakeholders	• Patient groups • FDA policy and review division staff • SMEs, disease experts
Enablers / Requirements	EL-PFDD are convened and executed by patient groups in collaboration with their key stakeholders. For FDA attendance at these meetings, FDA recommends submitting a Letter of Intent (LOI) to FDA approximately 1 year before the anticipated meeting date. The LOI should be no more than 5 pages and communicate the importance of the meeting in the context of the disease area, and important details regarding the meeting plan. • Guidelines are available, see below for some examples but access the FDA webpage to ensure you review the latest version: Collecting comprehensive and representative input: https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM610442.pdf • Methods to identify what is important to patients & select, develop or modify fit-for-purpose clinical outcomes assessments: https://www.fda.gov/downloads/Drugs/NewsEvents/UCM620707.pdf Meeting materials from a public workshop on development PFDD guidance from December 2017 is available here: https://www.fda.gov/Drugs/NewsEvents/ucm574725.htm
Output	The main output from EL-PFDD meetings is "the Voice of the Patient" report, that summarizes the results of the meeting. Additional deliverables could include Webcast recordings, a meeting transcript, and meeting materials (agenda, slides). Links to completed EL-PFDDs are listed on FDA's website: https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm368342.htm Meeting information from the original 24 FDA-sponsored PFDDs are also publicly accessible on FDA's website and include a meeting summary, background materials and meeting recordings: https://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm347317.htm
Best time to apply and time window	For FDA participation in an EL-PFDD, patient groups are advised to engage with FDA at least 1 year in advance. An EL-PFDD may be convened at any time in the disease research process.

Expert tips	There is abundant information on past meetings and the PFDD model that are accessible on FDA's website, including summary reports, background documents, recordings and other information: https://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm347317.htm Patient groups may also contact FDA staff in the Patient Affairs Staff office for assistance in meeting planning: https://www.fda.gov/about-fda/office-clinical-policy-and-programs/patient-affairs-staff PROs: – Per use section above, provides a unique and detailed cross-section of the patients' voice for clinical manifestations, diagnosis, clinical trial design and outcome measure assessment and development. CONs: – Resource intensive with prolonged planning time (~1 year).