Orphan Drug Development Guidebook

Building Block U218

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	Patient/caregiver interactions – FDA Patient Affairs Staff (PAS)
References	https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/ucm589472.htm For Patients | FDA Learn About FDA Patient Engagement | FDA
Description	At the FDA, the Patient Affairs Staff (PAS) is devoted to supporting cross-cutting patient engagement activities across the FDA. Its mission is to coordinate and support patient engagement activities across FDA to facilitate awareness and collaboration with patients, their advocates and the FDA. Objectives include: 1. Lead cross-center programs and activities across the medical product centers to inform regulatory work 2. Create and assist with public and private collaborations and partnerships to discuss regulatory topics of interest 3. Strengthen bidirectional communication through education and events Enhance the FDA's external communication platforms to expand public awareness and help patients, caregivers, and advocates navigate FDA engagement activities and the regulatory process.

	PAS can be contacted for any disease- or medical product-related reason and has frequent interactions with rare disease communities. It is likely that many rare diseases groups will find PAS interactions and programs useful.
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	The goal of this building block is to raise awareness in the patient and advocacy community on opportunities for engagement, support, communication and transparency with the FDA. PAS provides a venue for contacting FDA about medical product issues.
Stakeholders	• FDA PAS and other patient engagement groups throughout FDA product review centers, CBER, CDER, CDRH • Patients and advocates • Members of the public • Other US government agencies • Non-US regulatory agencies
Enablers/ Requirements	Membership Criteria: • Patients who have personal disease experience • Caregivers who support patients, such as a parent, child, partner, other family member, or friend, and who have personal disease experience through this caregiver role • Representatives from patient groups who, through their role in the patient group, have direct or indirect disease experience
Output	Output will vary depending on the questions and concerns of the patients and advocates contacting PAS. FDA will respond to questions or issues put before them, and provide materials and resources currently available (e.g., guidance, policy) to the public.

Best time to apply and time window	Anytime throughout the lifecycle of medical product development and regulatory decision-making for matters under FDA's purview.
Expert tips	PAS may also be contacted by: Online webform: Patients: Ask FDA Email: patientaffairs@fda.hhs.gov Phone (in US): 301-796-8460 PROs: – FDA staff devoted entirely to facilitating and enhancing patient and advocacy communication with FDA. Can be contacted at any time and for any disease or product related area.