Orphan Drug Development Guidebook

Building Block U225

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	Single Patient Expanded Access
References	https://www.fda.gov/downloads/drugs/guidances/ucm351261.pdf https://www.fda.gov/ucm/groups/fdagov-public/@fdagov-drugs-gen/documents/document/ucm351264.pdf
Description	This FDA program allows early access to drugs and medical devices before marketing approval, under regulatory oversight with collection of safety data, to individual patients with serious or immediately life-threatening diseases or conditions who lack therapeutic alternatives. This includes emergency and non-emergency expanded access. The Program allows individual patients with serious rare diseases to have access to pre-market drugs and devices, if the Sponsor agrees to this under their expanded access policy.
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Applicants developing medicines for rare and non-rare diseases. More specifically to: – treating physicians with agreement from the Sponsor

	– patients and families interested in access to pre-market drugs
Scope of use	• when a drug has been withdrawn for safety reasons, but there exists a patient for whom the benefits of the withdrawn drug continue to outweigh the risks • use of a similar, but unapproved drug (e.g., foreign-approved drug product) to provide treatment during a drug shortage of the approved drug • use of an approved drug where availability is limited by a risk evaluation and mitigation strategy (REMS) for diagnostic, monitoring, or treatment purposes, by a patient who cannot obtain the drug under the REMS; or • use for other reasons.
Stakeholders	• Sponsors • Patients/guardians • Physicians • FDA
Enablers / Requirements	• Sponsor must agree to provide drug • Physician must file an IND agree to prescribe the drug, and report adverse events per protocol (see notes on emergency use below) • Informed consent from the patient/guardian must be obtained
Output	Provision of care under approved expanded access protocol.
Best time to apply and time window	When a physician wants to submit a Single Patient Expanded Access request to obtain an unapproved investigational drug for an individual patient, he or she must first ensure that the manufacturer is willing to provide the investigational drug for expanded access use. If the manufacturer agrees to provide the drug, the physician should follow the steps below to submit an Investigational New Drug Application (IND) to the FDA.
Expert tips	• Physician may file an individual patient IND • Sponsor may submit a protocol for individual patient use to an existing IND • FDA may authorize expanded access for an individual patient without a written submission if there is “an emergency that requires the patient to be treated

	before a written submission can be made." In this case, expanded access to the drug may begin upon authorization (usually provided by telephone or other rapid means of communication) by the reviewing FDA official. The licensed physician or sponsor, however, must agree to submit an expanded access IND or protocol within 15 working days of FDA's authorization of the use. • In the case of an emergency expanded access, it is not necessary to wait for IRB approval to begin treatment. However, the IRB must be notified of the emergency expanded access use within 5 working days of emergency use. PROs: – Provides access to pre-market drugs for patients without therapeutic alternatives. CONs: – Requires safety monitoring, IRB oversight. – Cost of drug and study borne by Sponsor or, with FDA authorization, may be charged to a patient or third-party payor.