

Orphan Drug Development Guidebook

Building Block I404

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	Crowdfunding
References	<p>Multiple websites/platforms provide crowd funding services or describes how to initiate crowdfunding for rare diseases, e.g.,</p> <p>https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5818677/</p> <p>https://www.hcplive.com/view/amplify-hope-study-educates-families-on-crowdfunding-for-genetic-testing</p> <p>https://theprint.in/health/govt-sets-up-rare-disease-crowdfunding-portal-30-patients-get-26500-donations-in-a-week/716538/</p>
Description	<p>The internet and social networking have made crowdfunding a realistic avenue for individuals and groups hoping to fund medical and research causes, including patients in need of whole exome genetic sequencing (WES), as well as R&D projects.</p> <p>Traditionally, the main funding sources for rare diseases and orphan drug R&D are government, pharmaceutical companies, and venture capitalists.</p> <p>In recent years, crowdfunding is becoming a new funding source via social networks and the internet.</p>
Category	Funding Building Block
Geographical scope	International
Availability	Applicants developing medicines for rare and non-rare diseases.

Scope of use	To raise funding to fund rare disease diagnosis and R&D.
Stakeholders	<p>Two parties:</p> <ul style="list-style-type: none"> • stakeholders in rare disease diagnosis and R&D (Patients and families. Patient groups. Foundations, Researchers, or R&D companies), and • the public
Enablers/ Requirements	State the cause and justify the amount to be raised and the target raise amount.
Output	Funding
Best time to apply and time window	The best time to apply is at very early stage of R&D. While private funding is possible throughout the development life cycle, public funding usually lasts until the end of human PoC.
Expert tips	<p>Be as transparent as possible as to how the funds are used</p> <p>Do not misuse public donations.</p> <p>PROs:</p> <ul style="list-style-type: none"> – Leverage on public contribution – Promote public awareness <p>CONs:</p> <ul style="list-style-type: none"> – May not always meet the target raise amount – May not always meet the project timing (i.e., when you need the money to address the scientific question)