

## Orphan Drug Development Guidebook

### Building Block U226

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	NIH funded programs and resources
References	<a href="https://grants.nih.gov/grants/oer.htm">https://grants.nih.gov/grants/oer.htm</a>
Description	NIH is the largest public funder of biomedical research in the world, investing more than \$32 billion a year to enhance life, and reduce illness and disability.
Category	Development Opportunity Building Block
Geographical scope	United States of America
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	To provide funds for different phases of drug development.
Stakeholders	<ul style="list-style-type: none"> <li>• NIH,</li> </ul>

	<ul style="list-style-type: none"> <li>• Developer (public or SME)</li> </ul>
Enablers / Requirements	Enablers depends on each specific funding application.
Output	A successful funding application both results in a research proposal and the means to conduct the research.
Best time to apply and time window	The tool has its best use at very early phases of drug development
Expert tips	<p>Start applying for funding well in advance, and also consider alternative funding options.</p> <p>PROs:</p> <ul style="list-style-type: none"> <li>– Allows a substantial amount of funding, when obtained, especially for early phases of drug development.</li> </ul> <p>CONs:</p> <ul style="list-style-type: none"> <li>– High commitment is required from participants, while only a part of proposals gets granted.</li> </ul>