

## Orphan Drug Development Guidebook

### Building Block I410

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	NCATS and NHGRI Genetic and Rare Diseases Information Center (GARD)
References	<a href="https://ncats.nih.gov/gard">https://ncats.nih.gov/gard</a> <a href="https://rarediseases.info.nih.gov/">https://rarediseases.info.nih.gov/</a>
Description	<p>The Genetic and Rare Diseases Information Center (GARD) is a program of the National Center for Advancing Translational Sciences (NCATS) and is funded by two parts of the National Institutes of Health (NIH): NCATS and the National Human Genome Research Institute (NHGRI). GARD provides the public with access to current, reliable, and easy-to-understand information about rare or genetic diseases their families, health care providers, researchers, and the public in English or Spanish.</p> <p>It provides accurate, up-to-date information about ongoing research, symptoms, treatment options, and other details. Sources for GARD and other hard-to-find information include the National Library of Medicine, scientific conferences, support groups, and clinical trials and research.</p>
Category	Development Resource Building Block
Geographical scope	International

Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	Foundational information and links to data sources organized by disease. Access to the website and to find appropriate information by disease.
Stakeholders	<ul style="list-style-type: none"> <li>• Any Developer</li> <li>• NIH</li> <li>• National Center for Advancing Translational Sciences (NCATS) and National Human Genome Research Institute (NHGRI)</li> </ul>
Enablers / Requirements	Internet access to resources and information.
Output	Disease-specific information and resources
Best time to apply and time window	The tool is best to access early on in development to get full view of research and activities in the disease-specific space.
Expert tips	<p>If you can't find the information you need on the GARD website, call or write to GARD, and the Information Specialists will answer your questions.</p> <p>E-mail form: <a href="https://rarediseases.info.nih.gov/about-gard/contact-gard">https://rarediseases.info.nih.gov/about-gard/contact-gard</a>  Website: <a href="http://rarediseases.info.nih.gov/">http://rarediseases.info.nih.gov/</a></p>