

## Orphan Drug Development Guidebook

### Building Block I428

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	IRDiRC Recognized Resources
References	<a href="http://www.irdirc.org/research/irdirc-recognized-resources/">http://www.irdirc.org/research/irdirc-recognized-resources/</a> or doi: 10.1038/ejhg.2016.137
Description	<p>IRDiRC Recognized Resources is a quality indicator, based on a specific set of criteria that was created to highlight key resources, which, if used more broadly, would accelerate the pace of translating discoveries into clinical applications. Any resource compliant with the criteria may apply for the label. Selected platforms, tools, standards, and guidelines must be of fundamental importance to the international rare diseases research and development community.</p> <p>IRDiRC Recognized Resources is a quality indicator, which is expected to help accelerate the pace of translating discoveries into clinical applications. It therefore lists a number of resources important for Orphan Drug Development.</p>
Category	Development Resources Building Block
Geographical scope	International
Availability	Applicants developing medicines for rare diseases.

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Scope of use	This tool is mostly used to provide visibility to the different resources specifically relevant for the rare diseases field.
Stakeholders	<ul style="list-style-type: none"> <li>• Developers</li> <li>• Reviewers from the IRDiRC Scientific Committees and beyond</li> <li>• The users of these resources</li> </ul>
Enablers / Requirements	A working internet connection and access via PubMed.
Output	IRDiRC Recognized Resources criteria encourage long-term sustainability, peer review, and widespread relevance of the platforms, tools, standards, and guidelines. Applications for the label are accepted on a rolling basis and evaluated through a peer-review process by IRDiRC Scientific Committee members as well as experts in the relevant field. It is a public label which can be made visible on and by the resource, giving users an assurance of its quality and appropriateness.
Best time to apply and time window	The tool has its best use in the very early phases of the orphan drug pipeline.
Expert tips	<p>Look regularly through the list of IRDiRC Recognized Resources in order to be informed of new resources that have come around.</p> <p>PROs:</p> <ul style="list-style-type: none"> <li>– Freely accessible, easy to access.</li> </ul> <p>CONs:</p> <ul style="list-style-type: none"> <li>– Not a complete list, key resources might be missing, as resource holders need to apply themselves.</li> </ul>