

## Orphan Drug Development Guidebook

### Building Block I425

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	FAIR Principle for Data use
References	<a href="https://www.nature.com/articles/sdata201618">https://www.nature.com/articles/sdata201618</a> <a href="https://www.go-fair.org/fair-principles/">https://www.go-fair.org/fair-principles/</a>
Description	Lack of ultimate data use in rare disease created many silos slowing down development. The FAIR is coming to bridge this gap by proving essential guidelines for optimal data use.
Category	Regulatory Building Block
Geographical scope	International
Availability	Applicants developing medicines for rare diseases.
Scope of use	FAIR is trying to gap the scarcity and fragmentation of data in rare disease.
Stakeholders	<ul style="list-style-type: none"> <li>• Patient organizations</li> </ul>

	<ul style="list-style-type: none"> <li>• Academia</li> <li>• HCPs</li> <li>• Industry</li> <li>• Regulatory bodies</li> </ul>
Enablers / Requirements	Patient organizations can be the enablers by supporting the FAIR principles in their advocacy
Output	It is a set of principles that need to be followed when dealing with data.
Best time to apply and time window	The tool has its best use throughout the life cycle development.
Expert tips	<p>PROs:</p> <ul style="list-style-type: none"> <li>– Findable: easy to identify and find for both humans and computers, with metadata that facilitate searching for specific datasets,</li> <li>– Accessible: stored for long term so that they can easily be accessed and/or downloaded with well-defined access conditions, whether at the level of metadata, or at the level of the actual data,</li> <li>– Interoperable: ready to be combined with other datasets by humans or computers, without ambiguities in the meanings of terms and values,</li> <li>– Reusable: ready to be used for future research and to be further processed using computational methods. This requires adequate information about how the data were obtained and processed (provenance) and an appropriate license</li> </ul>