

## 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 Principles 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	<p>The FAIR principles provide guidelines to improve the Findability, Accessibility, Interoperability, and Reuse of digital assets. The principles emphasise machine-actionability because of the increasing reliance on computational support to deal with data as a result of the increase in volume, complexity, and creation speed of data.</p> <p>The lack of optimized data use in rare diseases has created missed opportunities and many silos, slowing down research and the development of medical products. The FAIR principles aim to help bridge this gap by proving essential guidelines for optimal data management and stewardship.</p>
Category	Regulatory Building Block
Geographical scope	International
Availability	Applicants developing medicines for rare diseases.

Scope of use	Improve on the current fragmentation of data in rare disease research.
Stakeholders	<ul style="list-style-type: none"> <li>• Patient organizations</li> <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, provides a framework for improving the infrastructure for supporting the reuse of scholarly data
Best time to apply and time window	The tool has its best use throughout the life cycle development where data is generated.
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>