

## Orphan Drug Development Guidebook

### Building Block U219

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	FDA Qualification Programs - Drug Development Tools (DDTs)
References	<a href="https://www.fda.gov/drugs/development-approval-process-drugs/drug-development-tool-qualification-programs">https://www.fda.gov/drugs/development-approval-process-drugs/drug-development-tool-qualification-programs</a>
Description	<p>The FDA Qualification programs, or Drug Development Tool (DDT) qualification programs provides a framework for interactions between FDA and DDT requestors to guide the collection of data to support a DDT’s prospectively specified context of use. The guidance describes the process for qualifying drug development tools intended for potential use, over time, in multiple drug development programs. DDTs are methods, materials, or measures that aid drug development.</p> <p>DDTs include, but are not limited to,</p> <ul style="list-style-type: none"> <li>• <b>Biomarkers: qualification-programs:</b> <a href="https://www.fda.gov/drugs/drug-development-tool-qualification-programs/cder-biomarker-qualification-program">https://www.fda.gov/drugs/drug-development-tool-qualification-programs/cder-biomarker-qualification-program</a></li> <li>• <b>Clinical outcome assessments (COAs):</b> <a href="https://www.fda.gov/drugs/drug-development-tool-qualification-programs/clinical-outcome-assessment-coa-qualification-program">https://www.fda.gov/drugs/drug-development-tool-qualification-programs/clinical-outcome-assessment-coa-qualification-program</a></li> <li>• <b>Animal models</b> for drug development under the Animal Rule: <a href="https://www.fda.gov/drugs/drug-development-tool-qualification-programs/animal-model-qualification-program">https://www.fda.gov/drugs/drug-development-tool-qualification-programs/animal-model-qualification-program</a></li> </ul> <p>This guidance provides a framework for interactions between the Center for Drug Evaluation and Research (CDER) and the entity proposing the DDT for qualification (the</p>

	submitter). It also explains the kinds of data that should be submitted to support qualification of a DDT and creates a mechanism for CDER's formal review of the data to ultimately qualify the DDT.
Category	Regulatory Building Block
Geographical scope	United States of America
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	<ul style="list-style-type: none"> <li>• To qualify and make DDTs publicly available for a specific context of use to expedite drug development and review of regulatory applications</li> <li>• To provide a framework for early engagement and scientific collaboration with FDA to facilitate DDT development</li> <li>• To facilitate integration of qualified DDTs in regulatory review</li> <li>• To encourage development of DDTs for contexts of use with unmet needs</li> <li>• To encourage the formation of collaborative groups to undertake DDT development programs to increase the efficiency and lessen the individual resource burden incumbent with DDT development</li> <li>• To encourage innovation in drug development</li> <li>• To create a shared learning environment for exchanging information on DDT development</li> </ul>
Stakeholders	A person, group, organization (including the federal government), or consortium that takes responsibility for and initiates a DDT qualification proposal.
Enablers / Requirements	The qualification process includes three submissions: the Letter of Intent (LOI), the Qualification Plan (QP), and the Full Qualification Package (FQP). Anyone interested in entering the qualification program should submit an LOI.
Output	The qualification is a conclusion that within the stated context of use, the DDT can be relied upon to have a specific interpretation and application in drug development and regulatory review. <b><u>Once qualified, DDTs will be publicly available to be used in any drug development program for the qualified context of use.</u></b> Additionally, the qualified DDT can be included in IND, NDA, or BLA submissions without needing FDA to reconsider and reconfirm its suitability as long as:

	<ul style="list-style-type: none"> <li>• There are no serious study flaws</li> <li>• There are no attempts to apply the DDT outside the qualified context of use</li> <li>• There are no new and conflicting scientific facts not known at the time the qualification was determined</li> </ul>
<p>Best time to apply and time window</p>	<p>As soon as possible during the early drug development phase when you are considering using not validated tools to investigate primary and surrogate endpoints.</p>
<p>Expert tips</p>	<ul style="list-style-type: none"> <li>• A DDT can be used in a drug development program without it going through the qualification programs and is therefore not mandatory. The use of non-qualified DDTs will be reviewed by the relevant FDA center(s), division(s), and office(s) as part of IND, NDA, or BLA reviews.</li> </ul> <p>PROs:</p> <ul style="list-style-type: none"> <li>• Having qualified DDTs that can be used by many sponsors helps optimize drug development and evaluation.</li> <li>• A DDT that has the potential to be used in multiple drug development programs may be considered for qualification. Qualification may reduce duplication of efforts, allow resource and information sharing, and facilitate regulatory acceptance of the DDT for future applications utilizing the same context of use.</li> <li>• These qualification programs promote a collaborative setting where multiple interested parties may pool resources and data to decrease cost, expedite drug development, and facilitate regulatory review. Increased public availability of qualified DDTs for specific contexts of use is anticipated to benefit the public health through (1) increased availability of effective drugs, (2) earlier access to medical therapies and (3) an enhanced knowledge of the drug under development.</li> </ul> <p>CONs:</p> <ul style="list-style-type: none"> <li>• DDT acceptance in the drug development and regulatory review process has previously been on a sponsor-by-sponsor, drug-by-drug basis. Drug sponsors seeking to use specific DDTs have typically developed enough data to justify its use only in that one setting. Generalized applicability of DDTs across varied drug development programs is often left undetermined thereby potentially limiting an expanded use of the tool.</li> <li>• The resources needed to develop a DDT for a more generalized use is often</li> </ul>

	<p>beyond the capabilities of a single entity. FDA encourages forming collaborative groups, such as a public-private partnership (PPP).</p>
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