

Discussion on the Qualified Biomarkers Program (CDER/CBER)

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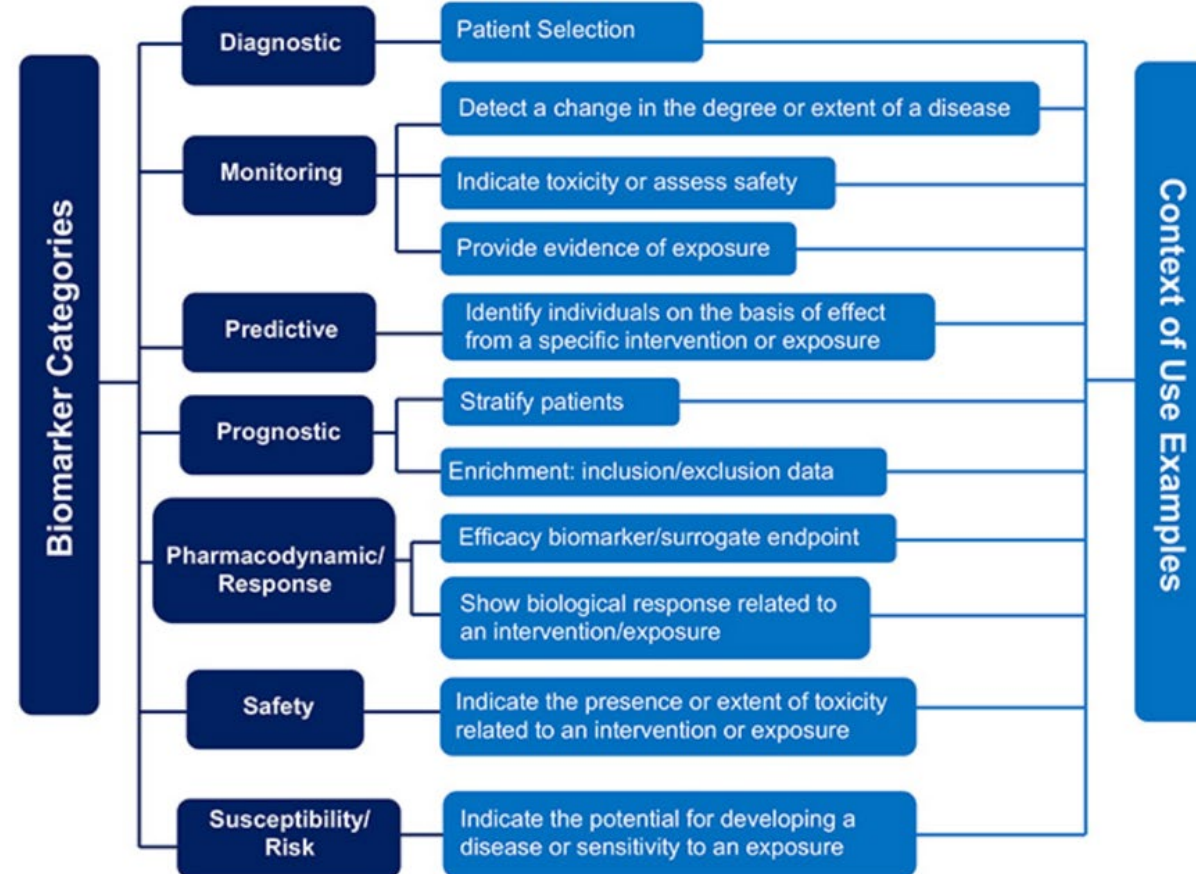
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What is a Biomarker?

- Definition – A "biological marker" that indicates a biological state or condition
 - Objectively measurable
 - Indicates normal or pathogenic processes or treatment responses.
 - Found in blood, urine, tissue, or other fluids
- Types & Applications:
 - Diagnostic – Detects and confirms disease presence
 - Prognostic – Predicts disease progression or tumor aggressiveness
 - Predictive – Forecasts patient treatment response
 - Pharmacodynamic/Response – Demonstrates biological response post-treatment

Best Biomarker Category and Examples of Context of Use

BEST Biomarker Category and Examples of Corresponding Drug Development Uses



Source: <https://www.fda.gov/drugs/biomarker-qualification-program/context-use>

Qualified Biomarkers

- Rigorously assessed for scientific and clinical relevance in drug development and review
- Uses:
 - Predict clinical outcomes
 - Identify suitable patients for treatments
 - Monitor treatment efficacy and safety
- Efficiency – Streamlines evaluation of new treatments
- Application – Used under its Context of Use (COU) in multiple drug development programs

FDA's Guidance on Biomarker Qualification

- Draft guidance – "Biomarker Qualification: Evidentiary Framework"
- Development recommendations under the 21st Century Cures Act
- Framework Components:
 - Drug development needs
 - Biomarker's COU
 - Benefit-risk assessment for drug development
- CBER works closely with CDER on drug development tools (DDTs) – a qualified biomarker is considered a DDT

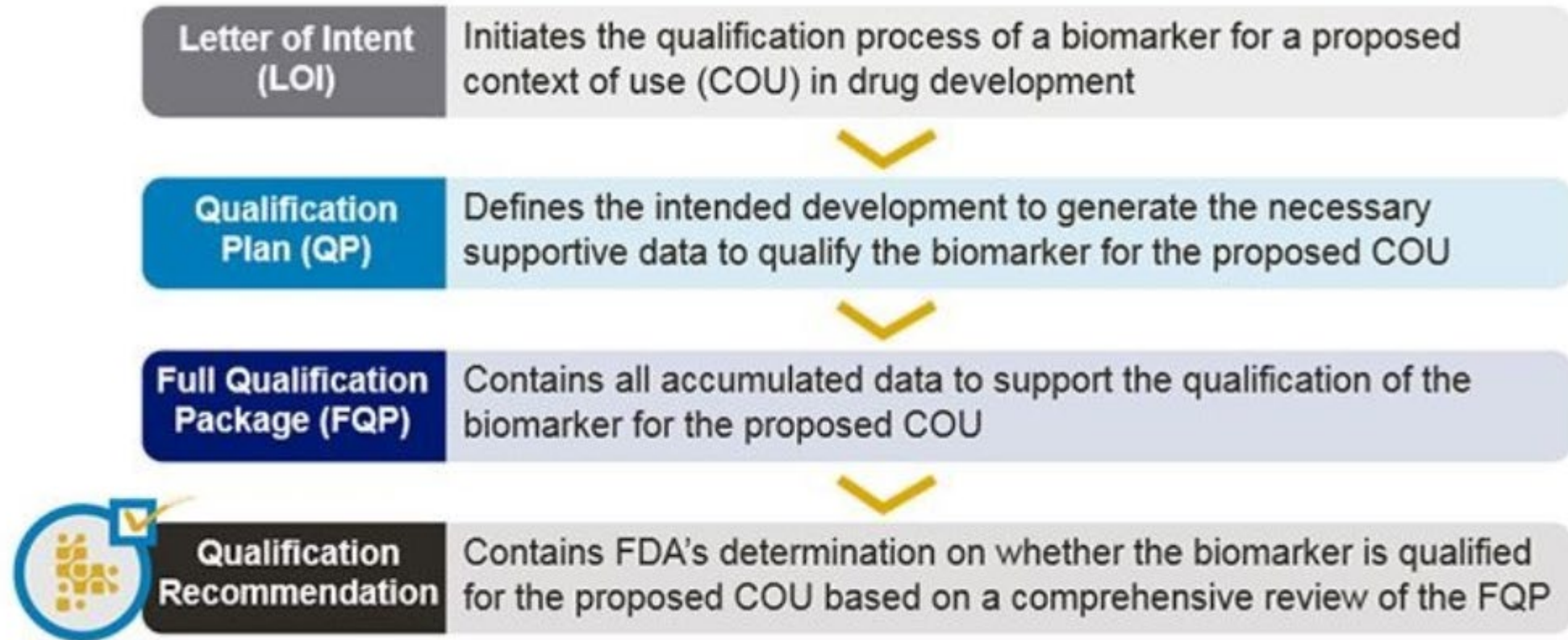
FDA's Biomarker Qualification Program (BQP)

- Encourages biomarker development across drug programs
- Approved biomarkers for:
 - Cancer, cardiovascular disease and neurological disorders
 - Examples:
 - HER2/neu (breast cancer)
 - EGFR (non-small cell lung cancer)
 - BRAF V600E (melanoma)
- CDER/CBER vs. CDRH's Programs
 - CDRH – Biomarker tests for medical device development
 - CDER/CBER – Focus on drug development
 - FDA-approved biomarkers for devices – e.g., cardiac troponin I (myocardial infarction diagnosis)

Interacting with CDER on Biomarkers

- Request a Pre-LOI Meeting with the BQP
- Letter of Support (LOS)
 - Overview – Indicates CDER's views on biomarker potential
 - Encourages further development
 - Doesn't imply a qualification
- Outside BQP – Engage via Critical Path Innovation Meetings (CPIMs)
 - The platform for discussing:
 - Early-phase biomarkers
 - Clinical outcome assessments
 - Innovative trial designs, etc.
 - Note - CPIMs are nonbinding

BQP Submission FAQs



Source: <https://www.fda.gov/drugs/biomarker-qualification-program/about-biomarkers-and-qualification>

BQP Submission FAQs – cont'd

- Pre-submission:
 - Ready to start?
 - See FDA's Context of Use (COU)
 - Request Pre-LOI Meeting
 - See FDA's Biomarker Qualification Letter of Intent (LOI)
 - Fees? None
 - Multiple biomarkers in one submission? Yes
- Submission Details:
 - Public information? Check Transparency Provisions
 - Can COU be modified during qualification? Yes
 - Reviewers? Primarily CDER and other FDA Centers as needed
 - Examples? Visit the FDA site's Biomarker Qualification Submissions
 - Can literature support qualification? Yes
 - More info? Email CDER-BiomarkerQualificationProgram@fda.hhs.gov.

Resources

- [FDA In Brief: FDA issues guidance to facilitate the efficient qualification of novel biomarkers that can help advance drug development | FDA](#)
- [Biomarker Qualification: Evidentiary Framework | FDA](#)
- [General Biomarker Information | FDA](#)
- [About Biomarkers and Qualification | FDA](#)
- [Pathways for Biomarker Integration in Drug Development | FDA](#)
- [Qualification Process for Drug Development Tools - Guidance for Industry | FDA](#)
- [Biomarker Qualification Program Submission Frequently Asked Questions | FDA](#)
- [Biomarkers - Toolkit | NIH](#)
- [Resources for Biomarker Requestors | FDA](#)
- [Letter of Support \(LOS\) | FDA](#)
- [Biomarker Qualification Letter of Intent \(LOI\) Template | FDA](#)
- [Context of Use | FDA](#)

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