U.S. FOOD & DRUG ADMINISTRATION

THE BEST RESOURCE: HARMONIZING BIOMARKER TERMINOLOGY

The Biomarkers, EndpointS, and other Tools (BEST) Resource glossary, www.ncbi.nlm.nih.gov/books/NBK326791, clarifies terminology and uses of biomarkers and endpoints as they pertain to the progression from basic biomedical research to medical product development to clinical care. It was developed by FDA and NIH to promote consistent use of biomarker terms and concepts, and thereby advance biomarker science.

BEST harmonizes terms and definitions and addresses nuances of usage and interpretation among various stakeholders. In addition, it includes examples of the various categories of biomarkers and their applications, and it distinguishes between biomarkers and clinical assessments.

Biomarkers are used in basic, translational, and clinical research and in clinical settings to facilitate medical product development and inform patient care decisions. Biomarkers even have applications in food safety, environmental science, veterinary medicine, and other fields. The scientific and medical communities, patients, providers, patient advocacy organizations, industry, and regulators all play important roles in the development and application of biomarkers.

Why was BEST developed?

Language confusion can hinder medical product development, causing misinterpretation of evidence, misunderstanding of evidentiary requirements, and even failure in late-phase trials.

In the spring of 2015, the FDA-NIH Joint Leadership Council identified the harmonization of terms used in translational science and medical product development as a priority need, with a focus on terms related to study endpoints and biomarkers. The FDA-NIH Biomarker Working Group, www.ncbi.nlm.nih.gov/books/NBK338449, a collaboration of representatives from multiple FDA Centers and NIH Institutes, published the BEST Resource in January 2016.

Using consistent, mutually understood terminology can help accelerate development, validation, and qualification of medical product development tools.

BEST efforts

Here is an example of how the definition of the term “biomarker” evolved as a result of the FDA-NIH Biomarker Working Group:

- **Initial definition of biomarker:** A characteristic that is objectively measured and evaluated as an indicator of normal biological processes, pathogenic processes, or pharmacologic responses to a therapeutic intervention.[1]
- **BEST definition of biomarker:** A defined characteristic that is measured as an indicator of normal biological processes, pathogenic processes, or responses to an exposure or intervention, including therapeutic interventions. Molecular, histologic, radiographic, or physiologic characteristics are types of biomarkers. A biomarker is not an assessment of how a patient feels, functions, or survives.

NIH and FDA encourage stakeholders to join them in using BEST terms and definitions so that everyone speaks the same language when discussing biomarkers and endpoints.

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We want your ideas

The BEST Resource is a living document that will be updated periodically with additional definitions and examples. All stakeholders—the scientific and medical communities, patients, providers, patient advocacy organizations, industry, and regulators—are encouraged to provide feedback on the glossary. Suggested revisions will be considered on a regular basis. To provide feedback, email biomarkers@ncbi.nlm.nih.gov.

Additional FDA biomarker resources

The following FDA resources provide more information about biomarkers and their role in medical product development:

Medical Product Development Tools at FDA
www.fda.gov/ScienceResearch/AboutScienceResearchatFDA/ucm468910

Drug Development Tools (DDT) Qualification Programs
www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram

Biomarker Qualification Program
www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm284076

Medical Device Development Tools (MDDT)
www.fda.gov/MedicalDevices/ScienceandResearch/MedicalDeviceDevelopmentToolsMDDT

1. Biomarkers Definitions Working Group. Biomarkers and surrogate endpoints: preferred definitions and conceptual framework. Clin. Pharmacol. Ther. 69, 89 -95 (2001).