Uncertainty in Benefit-Risk Assessment

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Problem Statement

To enhance **transparency** and **clarity**, a structured and **evidence-based** approach to **benefit-risk assessment** that characterizes **uncertainties** is desirable

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innovation that may improve health outcomes. Given the importance of these tasks, it is essential for FDA to adopt a harmonized framework for medical product approval that consistently accomplishes 3 key goals: (1) applying a core set of scientific, medical, and public health principles; (2) articulating a clear approach for addressing uncertainty and patient viewpoints; and (3) providing a predictable pathway for therapeutic development.

Robert Califf. Benefit-Risk Assessments at the US Food and Drug Administration: Finding the Balance. *JAMA.* 2017; 317:693-694

mean that the benefits outweigh the risks. Reviewers, however, must make this determination based on a tremendous amount of complex data, and must do so in contexts where there is a great deal of uncertainty. This uncertainty can stem from a variety of sources, including the nature of a given drug's benefits and risks, its effectiveness in a real-world population, and its long-term safety. For example, pre-market data are derived from randomized control trials (RCTs), which assess the efficacy of a drug in a highly controlled, narrow population that may not be representative of the wider population that may ultimately use the drug.[1] New information about potential harms or adverse events is gathered post-market, but there is uncertainty over how to reconcile results from observational studies with those from clinical trials.[2]

Advancing Structured Benefit-Risk Assessment in FDA Review
The Duke-Margolis Center for Health Policy, October 2017

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