

Guidance Snapshot

Benefit–Risk Assessment for New Drug and Biologic Products

Final Guidance for Industry



What Is Covered in This Guidance?

This final guidance provides an overview of the important considerations that factor into FDA’s benefit-risk assessment conducted during the regulatory review of new drug applications and biologics license applications and throughout the drug lifecycle. It also provides considerations about how sponsors can inform FDA’s benefit-risk assessment, through the design and conduct of the development program, as well as through the presentation of benefit and risk information in a drug product marketing application.



Why Is This Guidance Important?

At the FDA, the benefit-risk assessment is used to make an informed judgment as to whether the benefits (with their uncertainties) of the drug outweigh the risks (with their uncertainties and approaches to managing risks) under the conditions of use described in the approved product labeling. The benefit-risk assessment for a new drug can be straightforward in cases when a drug’s benefit is established as clinically meaningful and the drug’s safety profile is well-characterized with no serious risks identified. The benefit-risk assessment becomes more challenging in cases where there is greater uncertainty surrounding benefit or known or potential serious safety risks are identified, e.g., risks that are life-threatening or associated with significant morbidity.

Benefit-Risk Assessment Is Case-Specific and Considers:

THE EVIDENCE

submitted in the premarket application and/or generated in the postmarket setting that informs FDA’s understanding of the benefits and risks of the drug

THE THERAPEUTIC CONTEXT

in which the drug will be used, including the nature and severity of the condition the drug is intended to prevent, treat, cure, mitigate, or diagnose, and how well patients’ needs are being met by current available treatments

THE UNCERTAINTIES

about the drug’s benefits and risks. Although uncertainty can be reduced through careful study design and conduct, some uncertainty in the body of evidence available at the time of regulatory decision-making is inevitable, e.g., the frequency of rare serious adverse events or whether the drug’s effectiveness persists in long-term use.

THE REGULATORY OPTIONS

to reduce uncertainties and manage risks.

Specific Topics the Guidance Covers

Benefit-Risk Planning:

Benefit-risk planning by the sponsor, beginning early in development, can add value by helping to ensure that the clinical trial data and other supporting information collected are best suited to support the benefit-risk assessment. Benefit-risk planning is most valuable in cases where a challenging benefit-risk assessment can be reasonably anticipated. The goal of benefit-risk planning would be to direct drug development toward reducing important uncertainties and to increase the likelihood of establishing a favorable benefit-risk assessment of the submitted NDA or BLA.

Additional benefit-risk analyses to help inform the overall benefit-risk assessment may add value in some circumstances, such as decisions involving significant or unexpected uncertainties regarding the drug's benefits and risks. Consultation with FDA and careful planning early in drug development can increase the potential value of the benefit-risk analysis.

Interactions Between FDA & Sponsors:

FDA can provide insight and regulatory perspective that can inform a sponsor's benefit-risk planning appropriate to the issues identified at a particular stage of development. Thoughtful planning can also enhance the assessment of risk needed to support informed benefit-risk assessment. In particular, the discussions at an End of Phase 2 meeting can be particularly important when preclinical, early clinical, or other data identify a potential safety issue that would require greater certainty about the drug's benefits and/or risks to support approval.

Patient Experience Data:

Patients are experts in the experience of their disease or condition, and they are the ultimate stakeholders in the outcomes of medical treatment. Therefore, patient experience data, including patient preference information, can inform nearly every aspect of FDA's benefit-risk assessment throughout the drug lifecycle.

Presenting Benefit Risk Information:

The effective communication by sponsors of the drug's benefits, risks, and uncertainties is important to informing the benefit-risk assessment that supports regulatory decision-making.



To learn more about the benefit-risk assessment for human drug and biological products, [read the guidance](#).

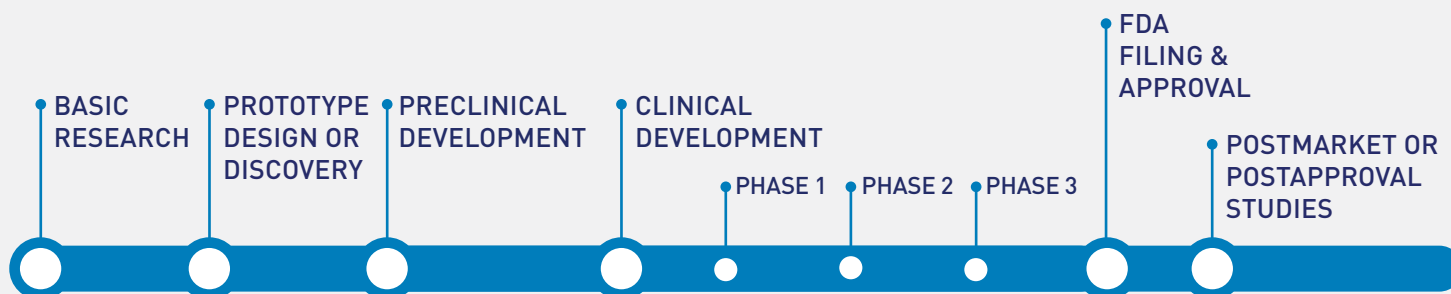
Background About the Guidance

Under the Federal Food, Drug, and Cosmetic Act (FD&C Act), for a drug to be approved for marketing in the United States, FDA must determine that the drug is effective and safe for use under the conditions prescribed, recommended, or suggested in the product's labeling. FDA interprets "safe" under the FD&C Act to mean the benefits of the drug outweigh its risks. The guidance provides considerations about the benefit-risk assessments that support FDA's regulatory decisions for new drug applications and biologic license applications.



Drug Development Timeline

The Guidance may be relevant to stakeholders throughout the drug development lifecycle



Guidance Recap Podcast

Speaker: Jim Smith, MD, Director of CDER's Office of New Drug Policy



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