NEW DRUG APPLICATIONS

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1 INTRODUCTION TO THE U.S. DRUG APPROVAL PROCESS

A new drug must be approved by the U.S. Food and Drug Administration (FDA) prior to marketing in the United States. "New drug" is defined by Federal Food, Drug, and Cosmetic Act (FDCA) § 201(p) as a human drug that is "not generally recognized . . . as safe and effective for use under the conditions prescribed, recommended, or suggested in the labeling" or one that, although recognized as safe and effective, has not been "used to a material extent or for a material time." The Supreme Court has concluded that a drug must have "adequate and well-controlled investigations"† of the drug's effectiveness to not be considered a new drug [1]. Because almost all drugs (unless already FDA approved) do not meet these criteria, they must go through the FDA drug approval process.

There are different approval processes for the different types of products that are regulated by the FDA. This chapter focuses on the application requirements for new human drugs, both through the new drug approval (innovator drugs) and abbreviated new drug approval (generic drugs) routes. The statutory provisions governing these requirements are found in the FDCA. Other drug application processes that will not be discussed include the over-the-counter review process, new animal drug applications, abbreviated new animal drug applications, and biologics license applications. The new drug application (NDA) process begins with evidence development to satisfy the conditions for approval. Clinical studies that are part of that evidence, if performed in the United States (and optionally if performed outside of the United States), require submission of an Investigational New Drug (IND) application.

2 INVESTIGATIONAL NEW DRUG APPLICATIONS

Prior to human clinical trials, preclinical investigation of a drug is performed in the laboratory and in animals to determine the drug's potential effects and to determine if the drug is safe for human testing. This testing typically includes both in vitro (in the laboratory outside of the body) and in vivo (in the body of a living organism) studies evaluating a drug's toxicity and pharmacology. FDA notification is not required prior to preclinical testing, but these investigations are subject to FDA regulations known as good laboratory practices. (See 21 CFR Part 58.)*

Commencement of human clinical trials in the United States require the submission of an IND application.

*This chapter was prepared by John McInnes in his personal capacity. The opinions expressed in this chapter are the author's own and do not reflect the view of the Centers for Medicare & Medicaid Services, the Department of Health and Human Services, or the United States government.

†Such investigations are also required for approval of a new drug application [FDCA § 505(d)].

*These regulations apply to the laboratory work and facilities that perform nonclinical studies intended to support a marketing application for FDA-regulated products, including drugs and biologics.

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