

Engaging with the FDA During New Drug Development

Glossary:

505(b)(1) Application - A 505(b)(1) application is an application that contains full reports of investigations of safety and effectiveness. The investigations the applicant relied on for approval were conducted by or for the applicant or the applicant has obtained a right of reference or use for the investigations.

505(b)(2) Application - A 505(b)(2) application is an application submitted under section 505(b) for which

- One or more of the investigations the applicant relied on for approval were not conducted by or for the applicant

and
- The applicant has not obtained a right of reference or use for the investigations (21 U.S.C. 355(b)(2))

Section 505(b)(2) expressly permits FDA to rely, for approval of an NDA, on data not developed by the applicant, such as published literature or the Agency's finding of safety and/or effectiveness of a previously approved drug product.

Biologic License Application (BLA): Biological products are approved for marketing under the provisions of the Public Health Service (PHS) Act. The Act requires a firm who manufactures a biologic for sale in interstate commerce to hold a license for the product. A biologics license application is a submission that contains specific information on the manufacturing processes, chemistry, pharmacology, clinical pharmacology and the medical affects of the biologic product. If the information provided meets FDA requirements, the application is approved and a license is issued allowing the firm to market the product.

Code of Federal Regulations (CFR): FDA's regulatory requirements, or codified portion of the final rule, are published under [Title 21 of Code of Federal Regulations](#).

End-of-Phase 1 (EOP-1) Meeting: When data from phase 1 clinical testing are available, the sponsor may again request a meeting with FDA-reviewing officials. The primary purpose of this meeting is to review and reach agreement on the design of phase 2 controlled clinical trials, with the goal that such testing will be adequate to provide sufficient data on the drug's safety and effectiveness to support a decision on its approvability for marketing, and to discuss the need for, as well as the design and timing of, studies of the drug in pediatric patients.

End-of-Phase 2 (EOP-2) Meeting: At specific times during the drug investigation process, meetings between FDA and a sponsor can be especially helpful in minimizing wasteful expenditures of time and money and thus in speeding the drug development and evaluation process. In particular, FDA has found that meetings at the end of Phase 2 of an investigation (end-of-Phase 2 meetings) are of considerable assistance in planning later studies and that meetings held near completion of Phase 3 and before submission of a marketing application ("pre-NDA" meetings) are helpful in developing methods of presentation and submission of data in the marketing application that facilitate review and allow timely FDA response.

FDA Safety and Innovation Act (FDASIA): Signed into law on July 9, 2012, FDASIA expands the FDA's authorities and strengthens the agency's ability to safeguard and advance public health by:

- Giving the authority to collect user fees from industry to fund reviews of innovator drugs, medical devices, generic drugs and biosimilar biological products;
- Promoting innovation to speed patient access to safe and effective products;
- Increasing stakeholder involvement in FDA processes; and
- Enhancing the safety of the drug supply chain.

Investigational New Drug Application (IND): An "investigational new drug" is a "new drug or biological drug that is used in a clinical investigation." The IND (investigational new drug application) is the vehicle through which a sponsor advances to the next stage of drug development known as clinical trials (human trials). An IND is a request for the Food and Drug Administration (FDA) authorization to administer an investigational drug to humans. Such authorization must be secured prior to interstate shipment and administration of any new drug that is not the subject of an approved new drug application.

New Drug Application (NDA): When the sponsor of a new drug believes that enough evidence on the drug's safety and effectiveness has been obtained to meet FDA's requirements for marketing approval, the sponsor submits to FDA a new drug application (NDA). The application must contain data from specific technical viewpoints for review, including chemistry, pharmacology, medical, biopharmaceutics, and statistics. If the NDA is approved, the product may be marketed in the United States. For internal tracking purposes, all NDAs are assigned an NDA number.

New Molecular Entity (NME): A New Molecular Entity is an active ingredient that has never before been marketed in the United States in any form.

Prescription Drug User Fee Act (PDUFA): PDUFA was created by Congress in 1992 and authorizes FDA to collect fees from companies that produce certain human drug and biological products. Since the passage of PDUFA, user fees have played an important role in expediting the drug approval process.

Special Protocol Assessment (SPA): In conjunction with the reauthorization of the Prescription Drug User Fee Act of 1992 (PDUFA) in November 1997, FDA agreed to specific performance goals (PDUFA goals) for special protocol assessment and agreement. The PDUFA goals for special protocol assessment and agreement provide that, upon request, FDA will evaluate within 45 days certain protocols and issues relating to the protocols to assess whether they are adequate to meet scientific and regulatory requirements identified by the sponsor. Three types of protocols related to PDUFA products are eligible for this special protocol assessment under the PDUFA goals: (1) animal carcinogenicity protocols, (2) final product stability protocols, and (3) clinical protocols for phase 3 trials whose data will form the primary basis for an efficacy claim if the trials had been the subject of discussion at an end-of-phase 2/pre-phase 3 meeting with the Review Division, or in some cases, if the division agrees to such a review because the division is aware of the developmental context² in which the protocol is being reviewed and the questions are being answered. The clinical protocols for phase 3 trials can relate to efficacy claims that will be part of an original new drug application (NDA) or biologics license application (BLA) or that will be part of an efficacy supplement to an approved NDA or BLA.