## OFFICE OF NEW DRUGS

# Developing Indication-Specific Guidances

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## PURPOSE

- This MAPP describes the process to be used by office and review division staff within the Office of New Drugs (OND) in the Center for Drug Evaluation and Research (CDER) when developing indication-specific guidances for industry.

## BACKGROUND

- The OND has, for many years, issued numerous guidances for industry that suggest approaches for drug development for specific clinical indications (*indication-specific guidances*). There is a general perception within the Food and Drug Administration (FDA) and industry that these guidances are useful in assisting sponsors in designing efficient and cost-effective drug development programs to support marketing applications. However, there is also the perception that the FDA does not issue enough indication-specific guidances (i.e., there are numerous indications for which indication-specific guidances do not exist, and from which sponsors could benefit if guidances existed in these areas).

- This MAPP aids the development of indication-specific guidances by providing a process and a template.[1] The purpose of the template is to increase the number of published indication-specific guidances as well as increase the quality and utility of these documents. This goal has been identified by OND as a key Critical Path initiative to facilitate drug development.

[1] The Indication-Specific Guidance Template can be found on the Guidance on Guidance ($G^2$) Web site at http://cdernet.cder.fda.gov/guidancedoc/gsquaredindex.htm.

## REFERENCE

- MAPP 4000.2 *Developing and Issuing Guidance* (http://www.fda.gov/cder/mapp.htm)

## DEFINITIONS

- **Indication-Specific Guidance:** A guidance for industry that suggests approaches for drug development for a specific clinical indication to support a marketing application.

- **Indication-Specific Guidance Template:** A structured outline and annotated table of contents that assists in the development of an indication-specific guidance. The Indication-Specific Guidance Template outlines the organization of content, promotes consistency of key elements in the document, and provides for easy retrieval of information from an indication-specific guidance. (The template can be found on the Guidance on Guidance ($G^2$) Web site at http://cdernet.cder.fda.gov/guidancedoc/gsquaredindex.htm.)

- **Therapeutic Product:** Refers to a drug or biologic product regulated in CDER that is developed to treat a particular indication or medical condition.

## PRINCIPLES

- The Indication-Specific Guidance Template should be used by all office and review division staff within OND to write guidances for developing therapeutic products for specific indications, with the exception of therapeutic products developed under the animal efficacy rule.

- The use of the Indication-Specific Guidance Template in developing indication-specific guidances is intended to support the fundamental principle of communicating to sponsors in a consistent, clear, and transparent manner, the Agency's current thinking on what constitutes a scientifically valid development program to support a marketing application for a specific indication. Guidances based on the template will provide advice that contains drug development best practices and regulatory precedents, and is driven by the most current scientific knowledge. In supporting this fundamental principle, this template emphasizes the need to address whether the resulting guidance document:
  - Is clear on what the FDA recommends the development program should look like and why; what the FDA considers the important development milestones to be and when relative to other steps they should be achieved; and how the FDA recommends development should proceed.

  - Identifies issues in the three dimensions of the Critical Path[2] that should be resolved to obtain approval for the specific indication in an efficient and timely manner.
  - Is written in plain language with appropriate definition of key technical terms.
  - Is brief, succinct, and contains only the scientific and regulatory recommendations needed to guide drug development (if the topic is a new therapeutic area, a more detailed guidance document may be appropriate, otherwise emphasis should be on brevity).

- During the development of an indication-specific guidance, the author should consider addressing in the guidance the following questions:
  - What are the potential challenges that the sponsor could face when developing the drug for the specific indication?
  - How can the sponsor meet those challenges?
  - When should the sponsor seek additional guidance from the review division?

- In this regard, an author should consider writing an indication-specific guidance when there is need to document the Agency's current thinking regarding what is known and what is unknown about drug development for a specific indication. This allows future interactions with the review division to focus on issues that are unique and specific to the investigational product.

- Authors and working groups should adhere to the template as much as possible to achieve consistency and completeness across indication-specific guidances. However, it is recognized that occasional modification of the template may be necessary to accommodate unique issues that arise from the development of a therapeutic product.

- Office and review division staff are to follow the conventions of the CDER Reviewer Style Manual when using the Indication-Specific Guidance Template. (The Reviewer Style Manual can be found on the $G^2$ Web site.)

## RESPONSIBILITIES AND PROCEDURES

- An individual **author** or an author in a **guidance working group** will write an indication-specific guidance based on the Indication-Specific Guidance Template available on the $G^2$ Web site. During guidance development, the author is responsible for engaging in scientific and regulatory dialogue with the appropriate subject matter experts both within and outside the Agency, as necessary, to develop complete and scientifically valid perspectives.

- The **author** should contact the OND immediate office (ondeio@cder.fda.gov) as early as possible in the guidance development process to obtain additional

[2] Assessment of Safety (how to predict if a potential product will be harmful); Proof of Efficacy (how to predict if a potential product will have medical benefit); and Industrialization (how to manufacture a product on a commercial scale with consistently high quality).

information and assistance in the development and clearance of an indication-specific guidance. Development of the guidance should adhere to established CDER guidance development policies and procedures, as described in MAPP 4000.2 *Developing and Issuing Guidance*.

- The **OND immediate office** will post a list of OND guidances under development on its intranet Web site and ensure that the author obtains appropriate input from any offices and centers that are affected by the guidance.

- **Division and office directors** responsible for regulating products for the proposed indication will promote consistent use of the Indication-Specific Guidance Template.

## EFFECTIVE DATE

- This MAPP is effective upon publication.