U.S. Food and Drug Administration

Drug Trials Snapshots

WHAT IS THE PURPOSE OF DRUG TRIALS SNAPSHOTS?

Drug Trials Snapshots provide consumers with information about who participated in clinical trials that supported the FDA approval of new drugs. The information provided in these Snapshots also highlights whether there were any differences in the benefits and side effects among sex, race and age groups. Drug Trials Snapshots is part of an overall FDA effort to make demographic data more available and transparent.

HOW TO USE SNAPSHOTS:

Each Snapshot includes contains information about the drug in a question and answer format. At the end of each section of the Snapshot, there is a shaded bar with the words “MORE INFO”. Click the “MORE INFO” bar for more technical and detailed content. At the bottom of each Snapshot, there is a link to the drug’s Package Insert as well as the medical review.

LIMITATIONS OF SNAPSHOTS:

The Snapshot is intended as one tool for consumers to use when discussing a drug’s risks and benefits with their physician. Do not rely on Snapshots alone to make decisions regarding medical care. Do not use Snapshots to substitute for advice from your health care professional. Conclusions regarding how effective and safe a drug is among different sex, race, and age groups cannot always be made, often because the numbers of patients in some groups are too limited to allow for meaningful comparisons to other groups and to the overall results.

VISIT DRUG TRIALS SNAPSHOTS:

www.fda.gov/DrugTrialsSnapshots

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Resources for You

In this section you will find resources that each of FDA's three main audience groups use most often to answer regulatory and drug-related questions. Audience-specific resources pages are focused on information that a specific type of user, such as a consumer, may want to find. It is not intended to be an all-inclusive list, but instead serves as a guide to key information. We appreciate feedback about what content you would find most useful on these resource pages.

RESOURCES AVAILABLE:

Consumer-friendly information

- Medication Guides
  Information for patients on how to safely use certain drugs

Approval, labeling, side effects, and safety information

- Drug Trials Snapshots
  Information about who participated in the clinical trials for new FDA approved drugs

- Drugs@FDA
  Information on FDA-approved brand name and generic drugs including labeling and regulatory history

- Drugs with Approved Risk Evaluation and Mitigation Strategies (REMS)
  REMS is a risk management plan required by FDA for certain prescription drugs, that uses tools beyond routine professional labeling to ensure that the benefits of the drug outweigh its risks.

- Index to Drug-Specific Information
  List of drugs for which safety alerts or other advisories have been issued

- New Molecular Entity and New Therapeutic Biological Product Approvals
  Novel new drugs approved by year

VISIT RESOURCES FOR YOU:

www.fda.gov/Drugs/ResourcesForYou

U.S. Food and Drug Administration

FDA Case Study

Drug Approval-Bringing a New Drug to the Market

A SMALL PHARMACEUTICAL COMPANY REVIEWS THE PATH LEADING TO FDA APPROVAL TO MARKET A NEW DRUG IN THE UNITED STATES

THE FDA CDER DRUG APPROVAL CASE STUDY IS A NEW LEARNING TOOL DESIGNED TO ADVANCE KNOWLEDGE, INSIGHT AND UNDERSTANDING OF FDA'S DRUG REGULATORY PROCESSES.

Who is it for?

- Students and health professionals interested in drug development (including medical and pharmacy students)
- Pharmaceutical and clinical innovators
- Small business staff
- Patients and patient advocacy groups

What does it cover?

The case study guides the reader through the steps of drug development and opportunities for interacting with FDA, including:

- Nonclinical testing
- Investigational New Drug application content and submission
- Clinical trials and protection of human subjects
- New Drug Application content
- FDA inspections of manufacturing and clinical sites
- Post marketing requirements

How can it be used in a classroom environment?

Students read the case study before class. Discussion questions are provided to emphasize the major points of the case study. Exercises, activities and quizzes to reinforce learning are provided.

Is continuing education credit offered?

CME is currently not offered for the case study.

How can patients benefit?

The case study helps patients understand how drugs are developed and approved, and how to engage with FDA through the drug approval process.

Where can I get it?

This case study may be used and distributed free-of-charge. It can be found at the FDA website [www.fda.gov/Training/ForHealthProfessionals/ucm464124.htm].

Sample text

"Green and her colleagues now faced the task of beginning the clinical trials needed to obtain approval from the US FDA to market the drug." "Can you give me a big picture overview of the drug development and approval process?" Soto asked.

Learning Objectives:

1. State the objectives of the drug development and approval process.
2. Identify the major activities that occur during the drug development and approval process from nonclinical tests through approval from the U.S. Food and Drug Administration (FDA).
3. Describe the major elements and steps to conduct a clinical trial.
4. Apply the drug approval process to a Fictional New Molecular Entity (NME) diabetes drug.

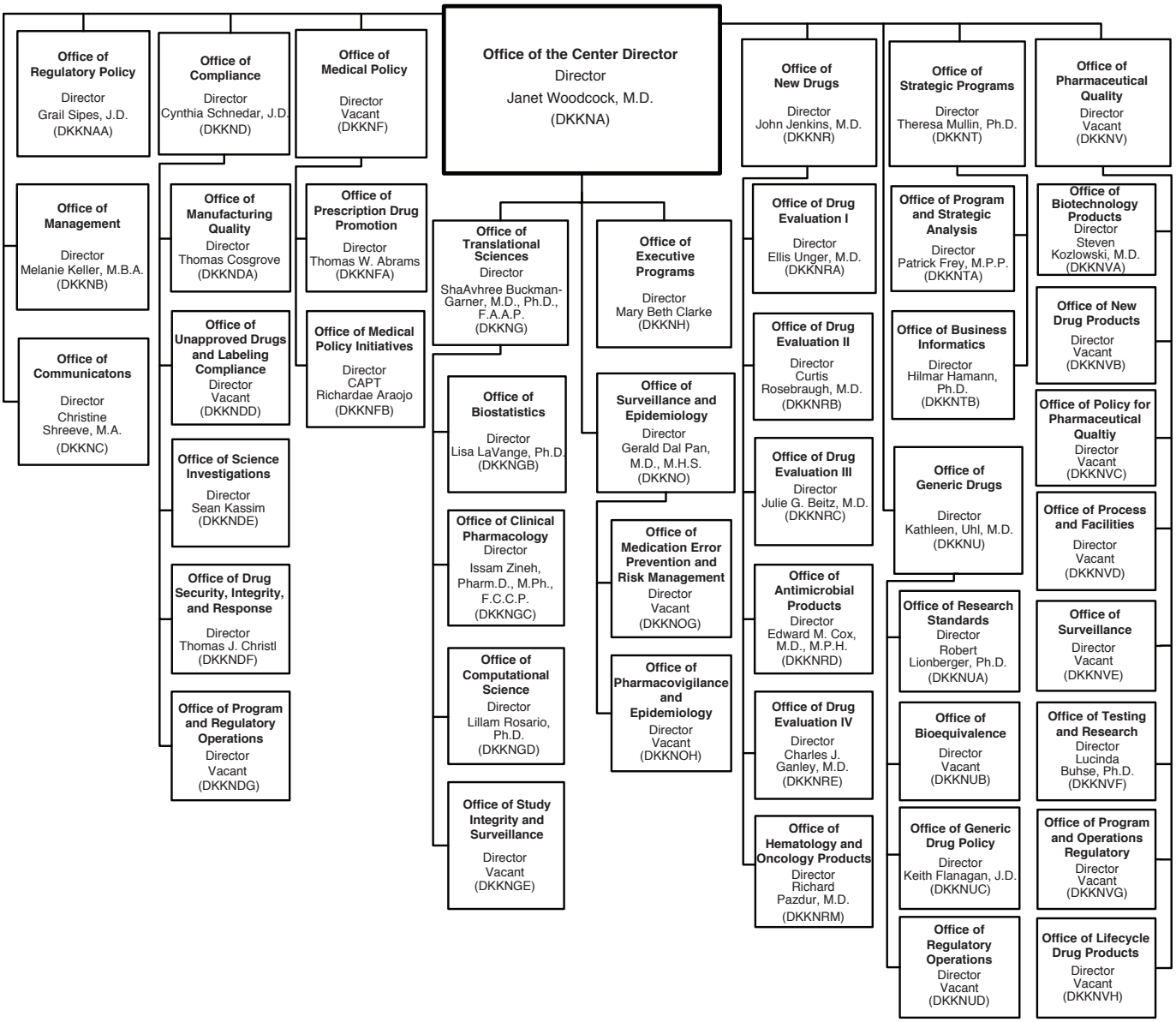
VISIT CDERLEARN TO VIEW THE FULL CASE STUDY:

http://www.fda.gov/TrainingForHealthProfessionals/ucm464124.htm

U.S. Food and Drug Administration

CDER Organizational Chart

Food and Drug Administration
Office of Medical Products and Tobacco
Center for Drug Evaluation and Research



VISIT CDER ORGANIZATION:

http://www.fda.gov/aboutfda/centersoffices/organizationcharts/ucm347877.htm

U.S. Food and Drug Administration

Professional Affairs & Stakeholder Engagement

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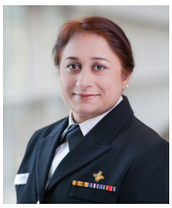

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U.S. Food and Drug Administration

Drug Approval Process

What is a drug as defined by the FDA?

A drug is any product that is intended for use in the diagnosis, cure mitigation, treatment , or prevention of disease; and that tis intended to affect the structure or any function of the body.

PRE-CLINICAL

Drug Sponsor's Discovery and Screening Phase

Drug Developed

Drug sponsor develops a new drug compound and seeks to have it approved by FDA for sale in the United States.

Animals Tested

Sponsor must test new drug on animals for toxicity. Multiple species are used to gather basic information on the safety and efficacy of the compound being investigated/researched.

IND Application

The sponsor submits an Investigational New Drug (IND) application to FDA based on the results from intial testing that include, the drug's composition and manufacturing, and develops a plan for testing the drug on humans.

IND REVIEW

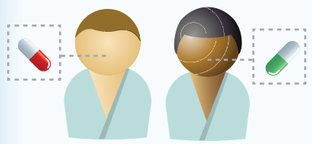
FDA reviews the IND to assure that the proposed studies, generally referred to as clinical trials, do not place human subjects at unreasonable risk of harm. FDA also verifies that there are adequate informed consent and human subject protection.

FDA's Center for Drug Evaluation and Research (CDER) evaluates new drugs before they can be sold.

The center's evaluation not only prevents quackery, but also provides doctors and patients the information they need to use medicines wisely. CDER ensures that drugs, both brand-name and generic, are effective and their health benefits outweigh their known risks.

CLINICAL

Drug Sponsor's Clinical Studies/Trials



PHASE 1

20-80

The typical number of healthy volunteers used in Phase 1; this phase emphasizes safety. The goal here in this phase is to determine what the drug's most frequent side effects are and, often, how the drug is metabolized and excreted.

PHASE 2

100's

The typical number of patients used in Phase 2; this phase emphasizes effectiveness. This goal is to obtain preliminary data on whether the drug works in people who have a certain disease or condition. For controlled trials, patients receiving the drug are compared with similar patients receiving a different treatment--usually a placebo, or a different drug. Safety continues to be evaluated, and short-term side effects are studied.

At the end of Phase 2, FDA and sponsors discuss how large-scale studies in Phase 3 will be done.

PHASE 3

1000's

The typical number of patients used in Phase 3. These studies gather more information about safety and effectiveness, study different populations and different dosages, and uses the drug in combination with other drugs.

Who reviews new drug submissions?

A team of CDER physicians, statisticians, chemists, pharmacologists, and other scientists review the drug sponsor's data and proposed labeling of drugs.

What other drug products are regulated by FDA?

Drugs include more than just medicines. For example, fluoride toothpastes, antiperspirants (not deodorant), dandruff shampoos, and sunscreens are all considered drugs.

NDA REVIEW

FDA's New Drug Application (NDA) Review

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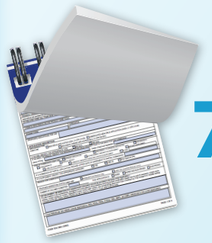
Drug Labeling

FDA reviews the drug's professional labeling and assures appropriate information is communicated to health care professionals and consumers.

8-9

Application Reviewed

After an NDA is received, FDA has 60 days to decide whether to file it so it can be reviewed. If FDA files the NDA, the FDA Review team is assigned to evaluate the sponsor's research on the drug's safety and effectiveness.



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NDA Application

The drug sponsor formally asks FDA to approve a drug for marketing in the United States by submitting an NDA. An NDA includes all animal and human data and analyses of the data, as well as information about how the drug behaves in the body and how it is manufactured.

6

Review Meeting

FDA meets with a drug sponsor prior to submission of a New Drug Application.

11

Facility Inspection

FDA inspects the facilities where the drug will be manufactured.

12 FDA

Drug Approval

FDA reviewers will approve the application or issue a response letter.

FASTER APPROVALS

The **Accelerated Approval** program allows earlier approval of drugs that treat serious diseases and that fill an unmet medical need. The approval is faster because FDA can base the drug's effectiveness on a "surrogate endpoint," such as a blood test or X-ray result, rather than waiting for results from a clinical trial.

The **Fast Track** program helps reduce the time for FDA's review of products that treat serious or life-threatening diseases and those that have the potential to address an unmet medical need. Drug sponsors can submit portions of an application as the information becomes available ("rolling submission") instead of having to wait until all information is available.

POST-MARKETING

FDA's Post-Approval Risk Assessment Systems

PHASE 4

Because it's not possible to predict all of a drug's effects during clinical trials, monitoring safety issues after drugs get on the market is critical. The role of FDA's post-marketing safety system is to detect serious unexpected adverse events and take definitive action when needed.

Once FDA approves a drug, the post-marketing monitoring stage begins. The sponsor (typically the manufacturer) is required to submit periodic safety updates to FDA.

www.fda.gov/medwatch
(800) FDA-1088 (322-1088) phone
(800) FDA-0178 (322-0178) fax

Food and Drug Administration MEDWATCH

FDA's MedWatch voluntary system makes it easier for physicians and consumers to report adverse events. Usually, when important new risks are uncovered, the risks are added to the drug's labeling and the public is informed of the new information through letters, public health advisories, and other education. In some cases, the use of the drug must be substantially limited. And in rare cases, the drug needs to be withdrawn from the market.

PDUFA

Prescription Drug User Fee Act

Since the PDUFA was passed in 1992, more than 1,000 drugs and biologics have come to the market, including new medicines to treat cancer, AIDS, cardiovascular disease, and life-threatening infections.

PDUFA has enabled the Food and Drug Administration to bring access to new drugs as fast or faster than anywhere in the world, all while maintaining the same thorough review process. Under PDUFA, drug companies agree to pay fees that boost FDA resources, and FDA agrees to time frames for its review of new drug applications.