How the FDA-Approval Process Helps Healthcare Providers and Consumers

The extensive FDA-approval process is undertaken in an effort to ensure that the efficacy, safety, and quality of a medicine are established before use by the general public. This process can take years to complete.

FDA-Approval Process[1,2]

1 Initial steps

Discovery and development, identification and basic research of new compounds, and preclinical research

2 Early testing

Continued laboratory investigation and animal testing to answer basic safety questions (prepare and submit an Investigational New Drug submission to allow initiation of human trials)

3 Assessment of effectiveness, safety, and dosing by studying 100s-1000s of patients

- Clinical research assessment of safety and efficacy in humans

Phase 1 Studies Several months	• **Safety and dosage** • Typically, **20–100** healthy volunteers or people with the condition studied	~70% of drugs move to next phase
Phase 2 Studies Several months to 2 years	• **Efficacy and side effects** • Up to **several hundred** people with the condition studied	~33% of drugs move to next phase
Phase 3 Studies 1 to 4 years	• **Efficacy and adverse reactions** • Typically, **300–3,000** people with the condition studied	~25%–30% of drugs move to submission for approval

When evaluating medicines for rare diseases, the FDA has approved medicines based on studies in which smaller numbers of patients were available to participate.

- Data from these studies are used to prepare a New Drug Application
- During this time, the FDA confirms a company's good manufacturing practices to ensure that it can provide consistent product

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4 The FDA examination of data

The FDA thoroughly examines all of the submitted data related to the drug to make a decision

5 Monitoring and studies continue after a product becomes available

Postmarket safety monitoring

The FDA monitors all drug and device safety once products are available for use by the public

Added efficacy and safety studies

After the FDA approves a new drug, Phase 4 studies can be conducted to provide more information

Phase 4 Studies	• **Safety and efficacy** • Typically, several thousand people who have the condition are studied

Following approval, the FDA continues to monitor the manufacturing to ensure that good manufacturing practices are maintained and ongoing product safety information is communicated to healthcare providers and patients

Additional testing for CNS medicines

For medicines that work through the central nervous system (CNS), developers are required to conduct studies to assess abuse potential and liability.[3] These studies help determine if a medicine could make a patient physically dependent and have the potential to be abused or misused.

When do drugs get expedited review?

For drugs that address an unmet medical need in the treatment of a serious or life-threatening condition, the FDA offers expedited programs to help ensure that therapies are reviewed and available to patients earlier when they have promise in treating such conditions.[4]

While medicines are being developed, can they be accessed by people not in clinical studies?

In some cases involving serious diseases or conditions, the FDA provides a pathway for physicians to request access to drugs for their patients while the drugs are being studied, before they are approved.[5] This is referred to as expanded access or compassionate use.

References: **1**. The FDA's drug review process: ensuring drugs are safe and effective. US Food & Drug Administration website. https://www.fda.gov/Drugs/ResourcesForYou/Consumers/ucm143534.htm. Accessed October 31, 2017. **2**. Step 3: clinical research. US Food & Drug Administration website. https://www.fda.gov/ForPatients/Approvals/Drugs/ucm405622.htm. Accessed January 23, 2018. **3**. Assessment of abuse potential of drugs: guidance for industry. US Food & Drug Administration website. https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm198650.pdf. Accessed January 23, 2018. **4**. Guidance for Industry: expedited programs for serious conditions—drugs and biologics. US Food & Drug Administration website. https://www.fda.gov/downloads/Drugs/Guidances/UCM358301.pdf. Accessed January 23, 2018. **5**. Expanded access: information for patients. US Food & Drug Administration website. https://www.fda.gov/ForPatients/Other/ExpandedAccess/ucm20041768.htm. Accessed January 23, 2018.

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