Drug and Vaccines: Development, Testing and Approval

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The Drug Development Process

Step 1: Discovery and Development
Research for a new drug begins in the laboratory.

Step 2: Preclinical Research
Drugs undergo laboratory and animal testing to answer basic questions about safety.

Step 3: Clinical Research
Drugs are tested on people to make sure they are safe and effective.

Step 4: FDA Review
FDA review teams thoroughly examine all of the submitted data related to the drug or device and make a decision to approve or not to approve it.

Step 5: FDA Post-Market Safety Monitoring
FDA monitors all drug and device safety once products are available for use by the public.
Clinical Drug Development

Phase 1

**Patience:** 20 to 100 healthy Volunteers.

**Length of Study:** Several months

**Purpose:** Safety and dosage

**Percentage of Drugs that Move to the next Phase:** 70%

Phase 2

**Patients:** Up to several hundred people with the disease/condition.

**Length of Study:** Several months to 2 years

**Purpose:** Efficacy and side effects

**Percentage of Drugs that Move to the Next Phase:** 33%
Clinical Drug Development

**Phase 3**

**Patients:** 300 to 3,000 volunteers who have the disease or condition

**Length of Study:** 1 to 4 years

**Purpose:** Efficacy and monitoring of adverse reactions

**Percentage of Drugs that Move to the Next Phase:** 25-30%

**Phase 4**

**Patients:** Several thousand volunteers who have the disease/condition

**Purpose:** Safety and efficacy
Post-Licensure Testing

• Lot testing

• Ongoing observation for rare events
  – With 1/5000 event, would take 250,000 subjects before get 95% confidence of seeing event

• VAERS
The Vaccine Approval Process for the United States

- Pharmaceutical company presents pre-clinical and clinical data to the US Food and Drug Administration (FDA)
- Center for Biologicals Evaluation and Research (CBER) is section of FDA in charge of vaccines
- CBER may ask the Vaccine and Related Biological Advisory Committee (VRBAC) to give opinion of the data
- CBER decides whether to license vaccine
Recommended Use of Approved Vaccines in the United States

• CDC reviews and recommends use
  – Advisory Committee on Immunization Practices (ACIP)
  – CDC Departmental Approval
  – Dept Health Human Services approval
  – Publication of policy in MMWR
FDA, Alternative Review Process

- **Fast Track**: Fast track is a process designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet medical need. [Fast Track]
- **Breakthrough Therapy**: A process designed to expedite the development and review of drugs which may demonstrate substantial improvement over available therapy. [Breakthrough Therapy]
- **Accelerated Approval**: These regulations allowed drugs for serious conditions that filled an unmet medical need to be approved based on a surrogate endpoint. [Accelerated Approval]
- **Priority Review**: A Priority Review designation means FDA’s goal is to take action on an application within 6 months. [Priority Review]