Path to Approval

Developing a Rare Disease Therapy in the United States

People living with rare disease play a critical role by:
- Educating others about the impact of a disease
- Communicating what’s important to them in a new therapy
- Contributing to clinical trial design
- Participating in clinical studies as volunteers

The medicine’s quality, identity, purity, and strength are confirmed for each batch.

Steps of Drug Development

Discoveries
- Researching possible new methods to treat disease
- Developing or discovering new molecules, technology, or new uses for existing medicines

Nonclinical Testing
- Determines safety and shows effectiveness in the target disease
- Animal testing obtains safety data about effects on multiple internal organs and proves potential efficacy of the compound

Phase 1
- Tests the safety of the medicine
- The safety is carefully tested in healthy volunteers, people with kidney or liver problems and sometimes in people with the rare disease.
- Even more studies seek information about how and when the medicine reaches specific body tissues

Phase 2
- More studies in people living with the rare disease
- The control group may receive a placebo (an inactive medicine) or the current standard treatment
- Tests if the medicine works in people with the condition, determines potential side effects and best dose

Phase 3
- Compares participants receiving the new medicine to those in a control group
- The control group may receive a placebo (an inactive medicine) or the current standard treatment

Phase 4
- Post-marketing surveillance and pharmacovigilance
- Monitors the product for long-term safety and efficacy

Clinical Trials:
- Many research studies are done in people to evaluate a potential medicine's efficacy and safety

Key Things to Know
- The U.S. Food and Drug Administration (FDA) regulates medicines in the U.S.
- The steps of drug development exist to make sure medicines work and are safe.
- It can take 10-15 years for a medicine to go from a science lab to an approved treatment.
- Regulators recognize the need for rare disease therapies and the Orphan Drug Act of 1983 provides ways to speed up the process.
- Patients and families are important participants in drug development.

Manufacturing
- Drug developers must follow good manufacturing practices (GMP) during and after Phase 2 studies.
  - Why is GMP important?
    - Buildings, equipment and processes are properly designed, monitored and controlled.
    - FDA inspects manufacturing facilities, including ones that manufacture ingredients and the finished product.

Speeding up the Process
- Under certain circumstances, companies can apply for FDA programs that facilitate faster review.
  - Fast Track Designation
  - Breakthrough Therapy Designation
  - Accelerated Approval
  - Priority Review

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