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

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

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

Clinical Trials:

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

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
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

Approval

Phase 4

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

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

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

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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