The Drug Development Process

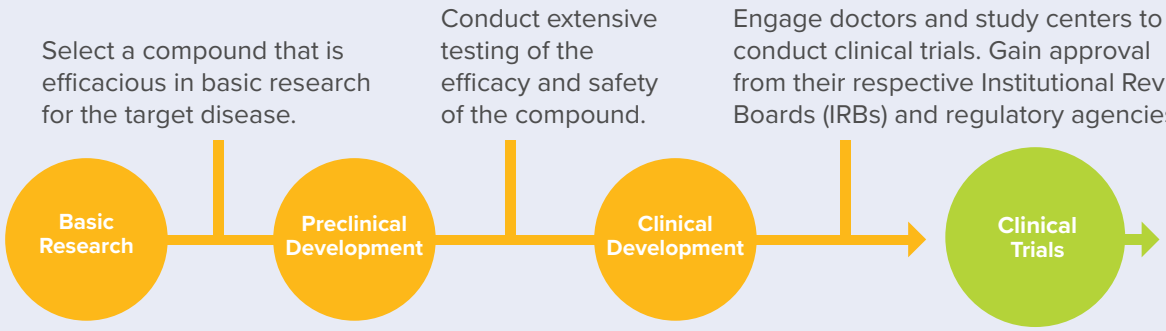
Patients and caregivers play an important role in the drug development process. This role is particularly vital in the development of orphan drugs where it can be especially difficult to bring new investigational therapies to market.

Barriers to Rare Disease Drug Development

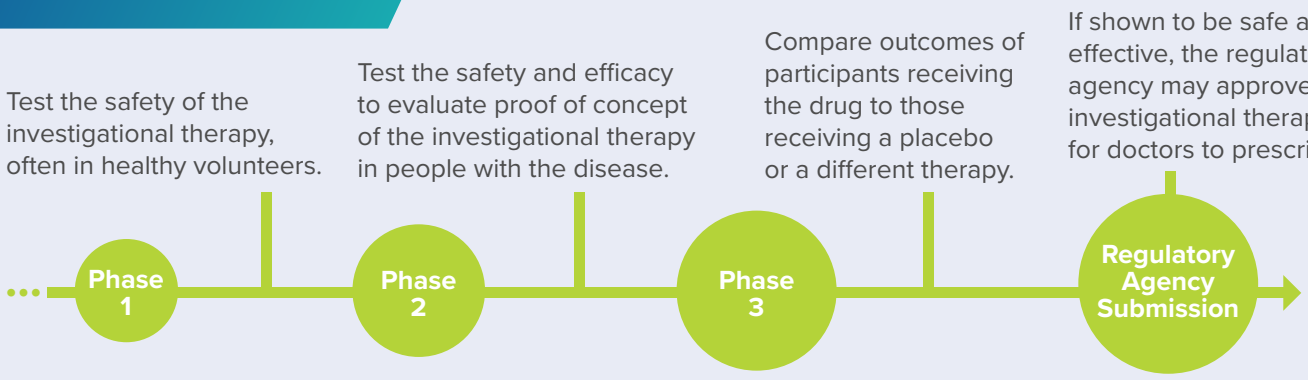
- Small and often diverse patient population
- Lack of awareness, education, and understanding of rare diseases
- Few medical experts

Once researchers identify a compound that has potential to benefit patients with a particular disease or condition, the development process begins.

Preclinical Trial Phases



Clinical Trial Phases



After Approval: The Fourth Phase of Clinical Research

Often referred to as “post-marketing monitoring,” the company continues to monitor the drug’s safety and effectiveness to keep track of long-term results.

Programs to Accelerate Approval

The Food and Drug Administration (FDA) in the U.S. and European Medicines Agency (EMA) in Europe have programs in place to help speed up the approval process of investigational therapies for diseases with high unmet medical need. Some examples include:

Fast Track Designation – Rolling reviews to help expedite the process.

Breakthrough Therapy Designation – Intensive guidance on efficient drug development and organizational commitment involving senior managers.

Accelerated Approval Pathway/Conditional Approval – Approval conditional on providing additional post-approval data. After confirmation, authorization is converted to a normal approval.

Approval Under Exceptional Circumstances – Approval with the understanding that complete data can never be provided.

Priority Review Designation/Accelerated Assessment – Reduced review time.

The drug development process requires a team working in tandem:

- **Researchers**
- **Physicians**
- **Academic centers**
- **Biopharmaceutical companies**
- **Patients**
- **Families**
- **Patient organizations**

The most powerful voice in the drug development process comes from the patient community.

References

Hall AK, Carlson MR. The current status of orphan drug development in Europe and the U.S. Intractable & Rare Diseases Research. *Intractable Rare Dis Res.* 2014; 3(1): 1-7. doi:10.5582/irdr.3.1.