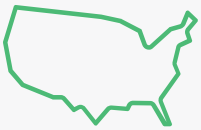
The Drug Development Process

Patients and caregivers play an important role in the drug development process, particularly in the orphan drug space where it is especially difficult to bring new drugs to the market. Once researchers identify a compound that could have a potential effect on a particular disease or condition, the process begins.

The drug development process is long, expensive, and requires a team working in tandem:

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

Typical amount of time it takes for medications to be approved:*

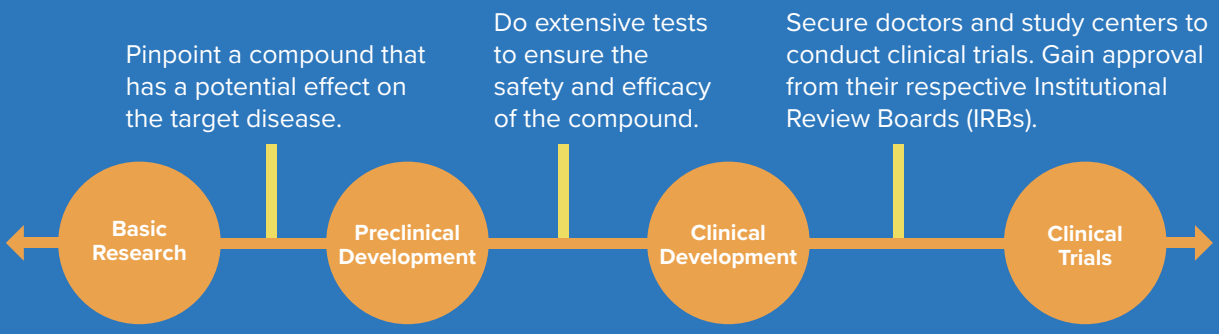


10 to 15 years in the U.S.

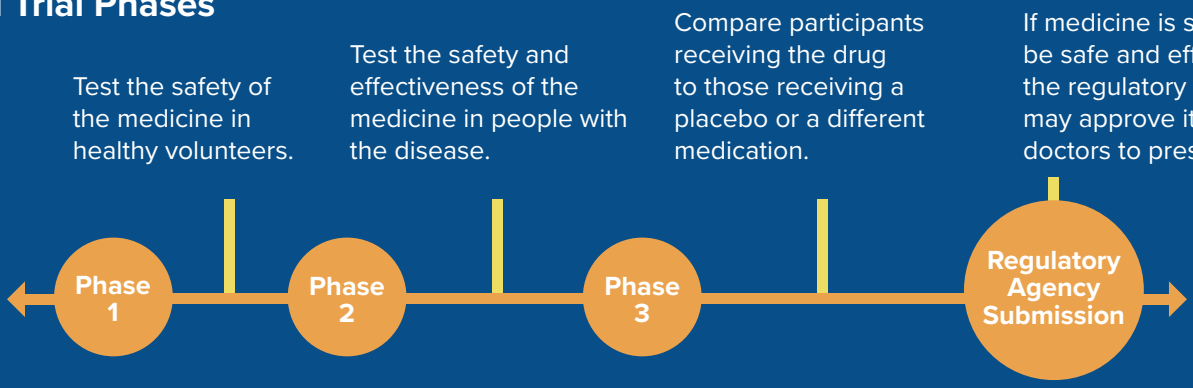
7 to 10 years in Europe

* without incentives from regulatory agencies to speed up the development process.[1]

Pre-Clinical Trial Phases



Clinical Trial Phases



After approval: the fourth phase of clinical study

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.

The Rare Advantage

Because patients with rare diseases often have no treatment options and need access to medicine as soon as possible, some drugs can qualify for special Food and Drug Administration (FDA) programs in the United States and European Medicines Agency (EMA) programs in Europe to help speed up the approval process for rare disease treatments.[2]

Program	Description	FDA	EMA
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 is 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 most powerful voice in the drug development process comes from the patient community.

Barriers to Rare Disease Drug Development

- Limited patient population
- Need for increased education and understanding of rare diseases
- Lack of awareness of rare diseases
- Few medical experts

References

[1] Desgrousilliers, M. Drug development time is money for some, life-and-death for others. ClinCapture. Available at http://www.clincapture.com/blog/drug-development-time-is-money-for-some-life-and-death-for-others/. Published May 24, 2012. Accessed April 25, 2018.

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