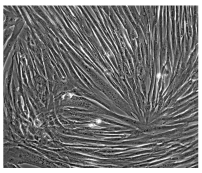
The path to a therapy

IND filing submitted to FDA.
(Investigational New Drug)

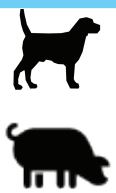
Drug Discovery & Development | **Preclinical Research** | **Safety & Toxicology** | **Clinical Trials** | **FDA Review** | **FDA Approval** | **Manufacturing Scale-up & Delivery**

Phase 1-3 — **0.5-2 yrs.**



In vitro cell culture

Mouse models



Large animal models

Nonhuman primates

Clinical trial volunteers

U.S. Department of Health and Human Services
Food and Drug Administration

Four methods to speed up the approval process:
- Fast track approval
- Breakthrough therapy
- Accelerated approval
- Priority review

Discover a new drug. — 12 years (US average) — Approved drug on the market.

Clinical Trials: Human subjects

FDA approval

Phase 0 (Pre-IND)	Phase 1	Phase 2a/2b	Phase 3	Phase 4
A microdose of a new drug is tested on a group of <15 subjects to see if the new drug behaves (pharmacokinetics/ pharmacodynamics) in human subjects as expected from preclinical studies.	Testing of an experimental drug on a group of 20 - 100 healthy volunteers to determine maximum dosing while producing minimal side effects.	The experimental drug is given to a group of up to ~200 patients. Doses are similar to Phase I; however, some participants may receive differing protocols. Phase 2a tests if the drug has a pharmacological impact on the disease. Phase 2b tests if the drug produces a meaningful change in the disease (clinical benefit).	Testing of an experimental drug on a group of 300 - 3,000 volunteers with disease to determine efficacy and for monitoring of adverse reactions.	Postmarket surveillance. Several thousand volunteers with disease and on the treatment are subject to continued monitoring for safety and efficacy.
Several days.	Several months.	Several months to 2 years.	1 - 4 years.	
Officially called an "exploratory IND study"	Repurposed drugs often skip Phase 1.			

Success rates vary. Overall, 9.6-11.8% of drugs entering Phase 1 get approval.

~70% move forward — **~31% move forward** — **~58% submitted for FDA approval; 85% approved**

Gene therapies combine Phases 1 and 2.

Most drugs fail at Phase 2.

Rare disease drugs have an overall ~25% success rate for getting FDA approval!

"Clinical Development Success Rates 2006-2015" by BioMedTracker and Amplion