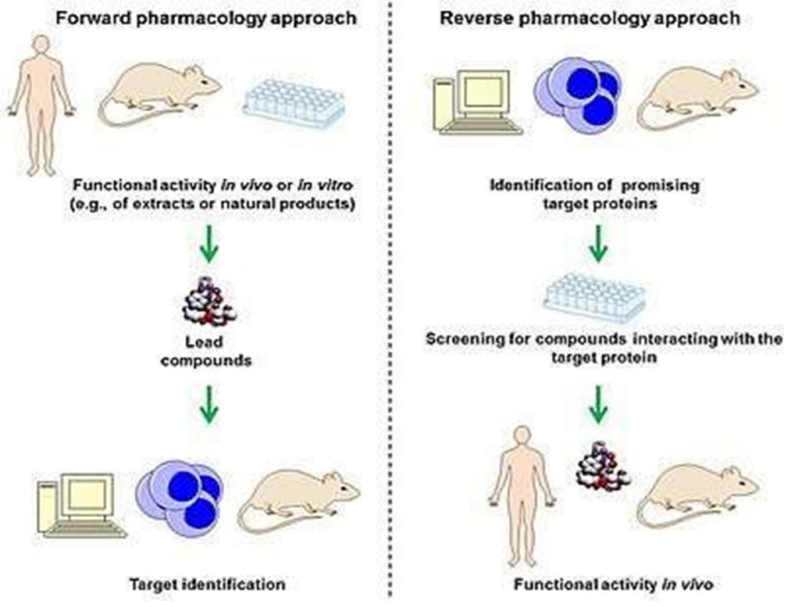
New drugs: development & evaluation



Developing drugs takes 12 years, however, in newer areas of medicine, like gene therapy, it can take up to 30 years. 12-30years

- 1 in 5000 new compounds are approved as pharmaceutical drugs by regulatory agencies like the Food and Drug Administration (FDA) in the US or the European Medicines Agency (EMA) in the EU.

Stage	Description	Key Activities	Duration	Outcome
Drug Discovery	Early research to identify potential drug candidates. Hits>>leads>>candidates	- Identify disease targets - Screen compounds (in silico testing). - Perform High-Throughput Screening (HTS) - Lead optimization to ensure specificity and potency.		- Discover lead candidates. - Identify 1-2 optimized drug candidates ready for preclinical testing.
Preclinical Research	Testing lead compounds in vitro, in vivo to assess the safety and toxicty	- In vitro testing (cell cultures). - In vivo testing (animal studies). - Safety tests to check for toxicity. - Application for (IND) in the US or (CTA) in the EU.	drug discovery with preclinical research take 4-7 years	- Most promising candidates (lead compounds) move to clinical trials. - Regulatory approval to start human testing.
Phase I -SAFETY	First trials in humans to evaluate safety, dosage, and pharmacokinetics (how the body absorbs and processes the drug).	- Test on **20-80** healthy volunteers. - Focus on safety, tolerability, and determining a safe dosage. -determining pharmacodynamics and pharmacokinetics	up to 1 year	- Safety profile in humans. - Optimal dosage range for further trials. Note :- women of childbearing age are excluded from trials
Phase II -PROOF OF CONCEPT	Trials to evaluate efficacy and further assess safety in patients with the target disease.	- Test on **100-300** patients who have the target disease. - Assess efficacy and safety. - Determine minimum effective dose and maximum tolerated dose. - Narrow down effective treatment regimens.	2 years	- Evidence of the drug's efficacy in treating the disease. - Refined dosage and safety data.

Phase III – REGULATORY EVIDENCE	Large-scale trials to confirm efficacy and safety on a broader population, gathering data for regulatory approval.	- Test on **1000** patients. - Monitor long-term side effects, drug interactions, and overall safety. - Data used for regulatory approval (NDA or MAA).	1-4 years	- Comprehensive data on drug's safety and efficacy. - Documentation for market approval submission (NDA in US, MAA in EU).
Market Approval	Submission of all trial data to regulatory bodies for review and potential approval.	- Prepare documentation for New Drug Application (NDA) or Marketing Authorization Application (MAA). - Includes data from discovery to Phase III trials. - Review by regulatory agencies (FDA, EMA) may take 6-10 months.	6-10 months	- If approved, the drug can be marketed. - Price negotiation with healthcare providers or insurers may vary by country.
Phase IV MONITORING,MARKETING AND SAFETY	Post-market surveillance to monitor long-term safety and efficacy once the drug is on the market.	- Ongoing monitoring of safety in real-world clinical practice. - Detects rare or long-term side effects not identified in earlier phases. - Assess interactions with other drugs. - Required for some drugs treating rare diseases. Assessed on complex medical conditions, drugs for the treatment of pregnant women, and for rare conditions	Ongoing (post-launch)	- Continual safety evaluation in a broader, real-world setting. - Pharmacovigilance: reporting additional safety or efficacy concerns.

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After completion of the preclinical tests, developers will apply for permission to proceed with clinical in-human studies. This is done either through an Investigational New Drug (IND) application in the US or a Clinical Trial Application (CTA) in the EU.

the drug registration process With good results from phases I-III, an application for market approval is submitted, called New Drug Application (NDA)*/Biologics License Application (BLA) in the US and Marketing Authorization Application (MAA) in the EU.
These can include hundreds of thousands of pages of documentation summarizing all collected data from the discovery phase onwards, and where the principal investigator argues for approval with the FDA and/or EMA

At this point, price negotiations begin between the principal and the potential buyers (government agencies or insurance companies, depending on the healthcare system). The price negotiation process can differ greatly from country to country.