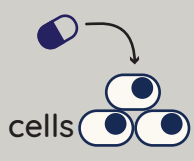
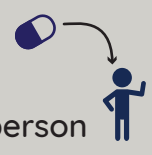
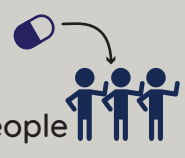
Stanford Medicine
COVID-19 Journal Club

Useful definitions for evaluating drug development studies

Preclinical Studies

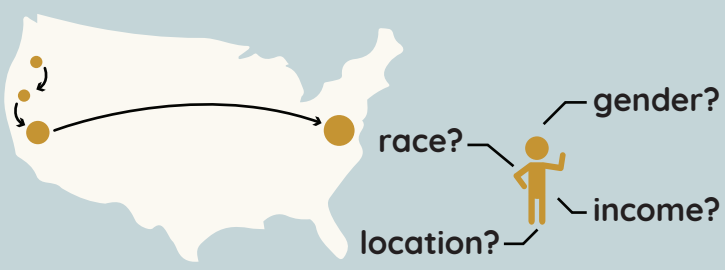
These studies are used to determine if a treatment might be effective in people and should then be tested in a clinical trial.

Type of preclinical study	In vitro experiments	Animal experiments	Case Report	Case Series
What is studied?				
What do we learn?	Whether a drug is safe and effective in cells & animals, and how or why it works		One patient's or multiple patients' experience(s) with the disease & treatment	
What CAN'T this study answer?	Effects in human subjects, which often differ greatly from preclinical results.		What would have happened to patients without the treatment	

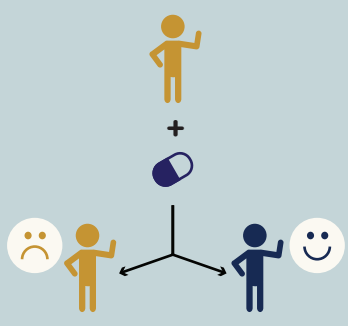
Clinical Studies

Studies in which scientists do not treat patients

Epidemiological Study
Study of where, when, and how frequently a disease is occurring, and who contracts it. For example, to see how a disease is transmitted.

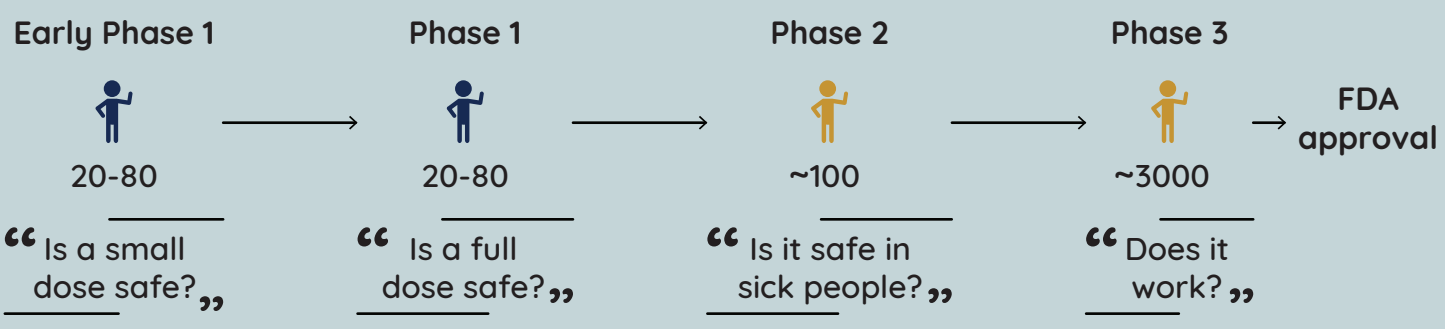


Observational Study
Study of how receiving a drug or having a disease affects health.



Studies in which scientists do treat patients

Clinical Trial
Study to determine whether or not a new treatment should be used in patients with a certain condition, or if a previously approved treatment should be used to treat a condition different than the one it was originally approved for.



Outcomes

Information collected to determine what effect a treatment is having on subjects. This tells us about the effectiveness of the treatment, and if there are severe side effects that could prevent its use.

Subjective Outcomes

How a patient feels

Objective Outcomes

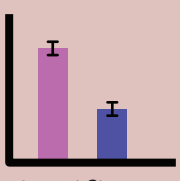
Vital Signs

Death or severe side effects

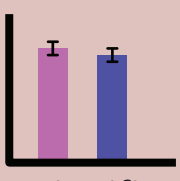
Virus in the blood

Statistical Significance

Based on how big and consistent a difference in outcomes is between subjects of different cohorts, researchers can calculate whether that difference is probably real (statistically significant) or happened by chance. Sometimes a study has too few subjects to confidently do this calculation.



significant



not significant

Clinical Significance

Would the treatment make a practically useful difference in patient care?

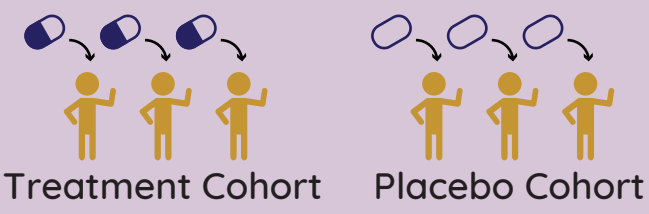
Features of High Quality Clinical Trials

Controlled

Researchers can compare a **cohort** of subjects who received the treatment with a **control** cohort. The controls may have gotten no treatment (**placebo**, or "sugar pill"), or an older method of treatment (**standard of care**).

Ideally, the only difference between cohorts is the treatment. But people have different age, sex, ethnicity, health history, and lifestyle habits. These factors can also affect their outcomes.

If cohorts are made up of subjects who equally represent these different characteristics, we can more reliably conclude that differences in outcomes are due to the treatment.



Randomized

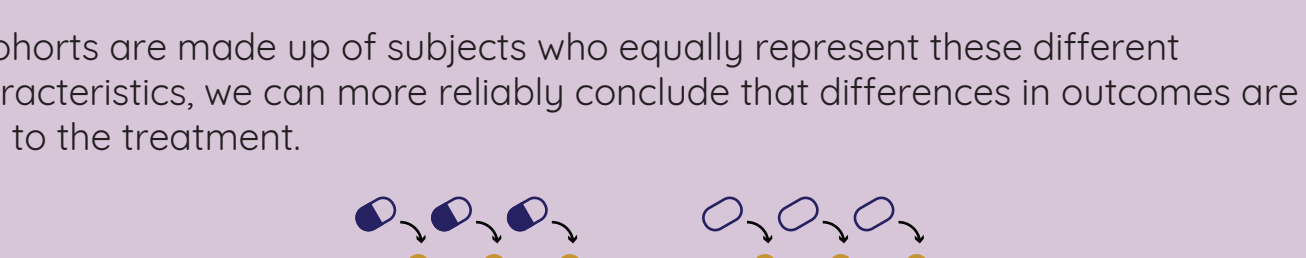
Randomization is when researchers randomly assign subjects to the treatment or placebo cohorts, as if by flipping a coin. This is the most reliable way to create cohorts that equally represent the different characteristics.

Blinded

In a **single-blinded study**, subjects are not told which treatment they are receiving.

In a **double-blinded study**, the researchers do not know either. **This is the most most rigorous method**.

In an **open-label study**, both subjects and researchers know. This is the least rigorous method because subjects' and researchers' behavior can influence outcomes.



"Learn About Clinical Studies." ClinicalTrials.gov, Mar. 2019, clinicaltrials.gov/ct2/about-studies/learn. Accessed 17 April 2020.

"NIH Clinical Research Trials and You: The Basics." National Institutes of Health, U.S. Department of Health and Human Services, 20 Oct. 2017, www.nih.gov/health-information/nih-clinical-research-trials-you/basics. Accessed 17 April 2020.

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