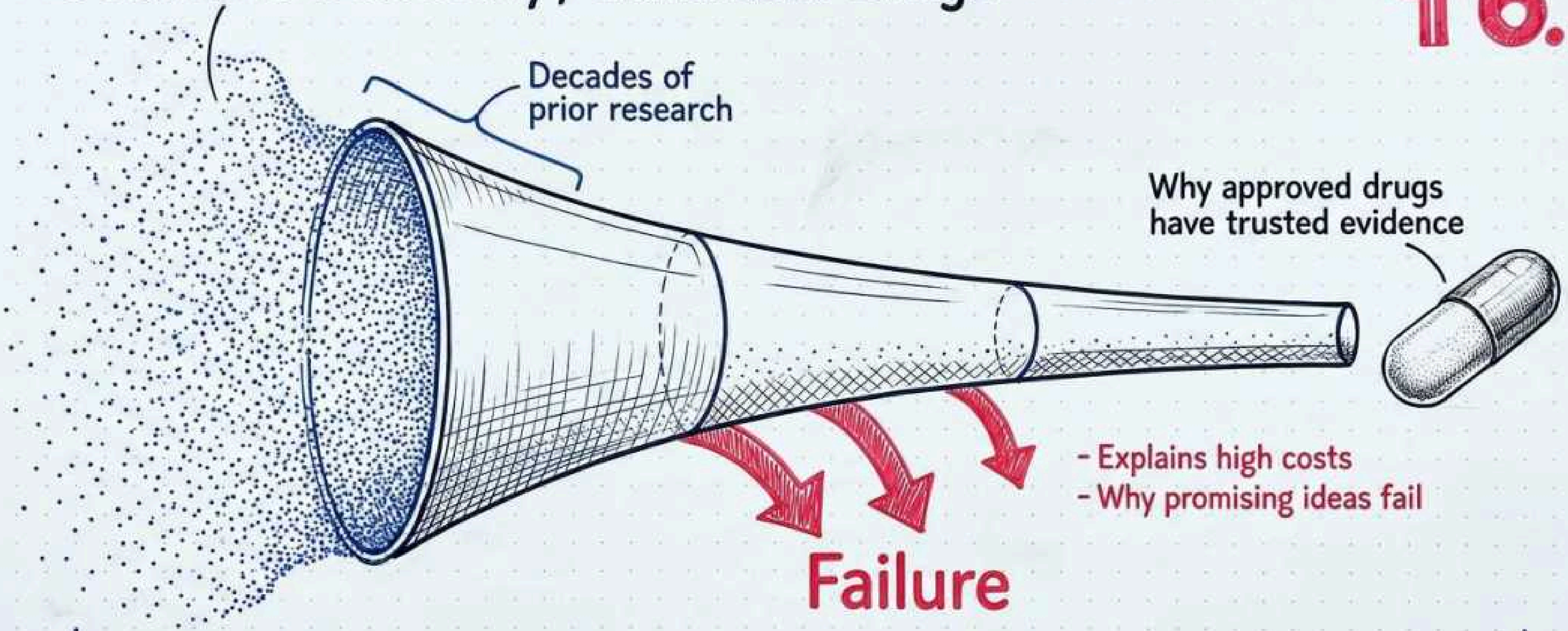
Scientific Discovery / Candidate Drugs

¶6.1

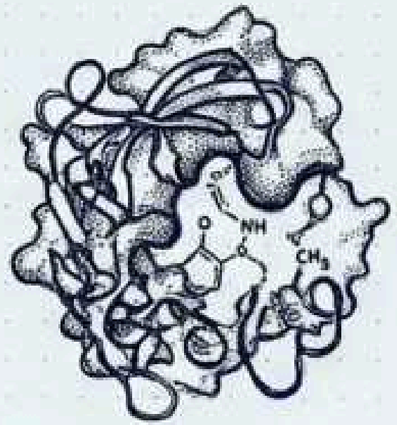


10 to 15 Years $

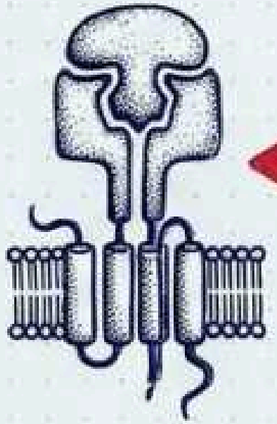
Gemini Notebook

¶6.2

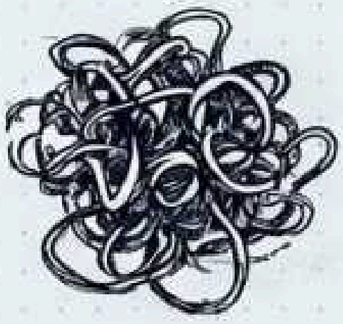
Enzyme Active Site



Cell Surface Receptor



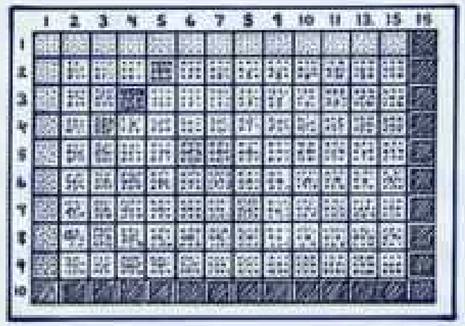
Faulty Signalling Protein Complex



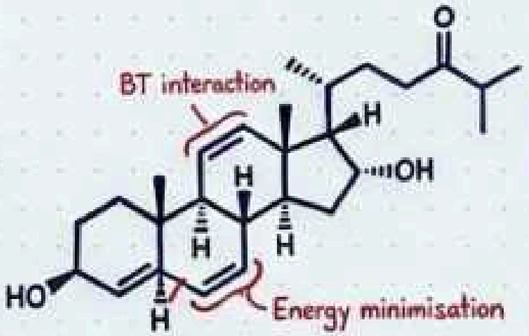


DNA Helix

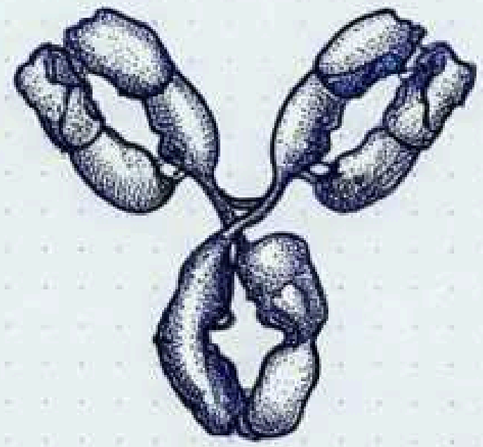
Genetics
(MED 503 B)
PCSK9 variants
(Cholesterol protection)



Screening



Rational Design

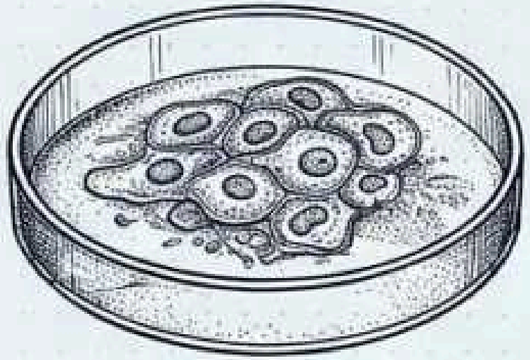


Biological Engineering

Gemini Notebook

¶6.3

In Vitro



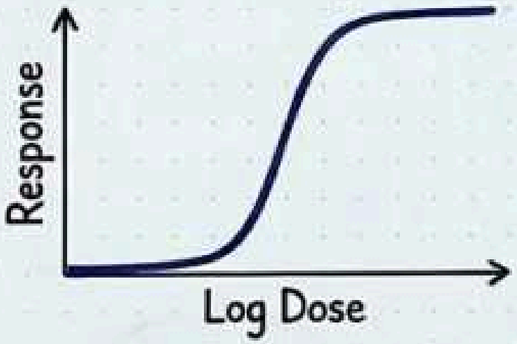
Refining binding & selectivity

In Vivo

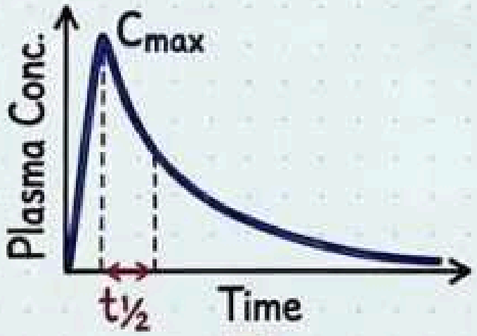


Preclinical Filter

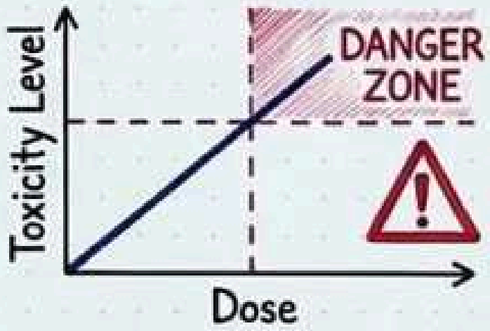
Efficacy



Pharmacokinetics



Toxicity

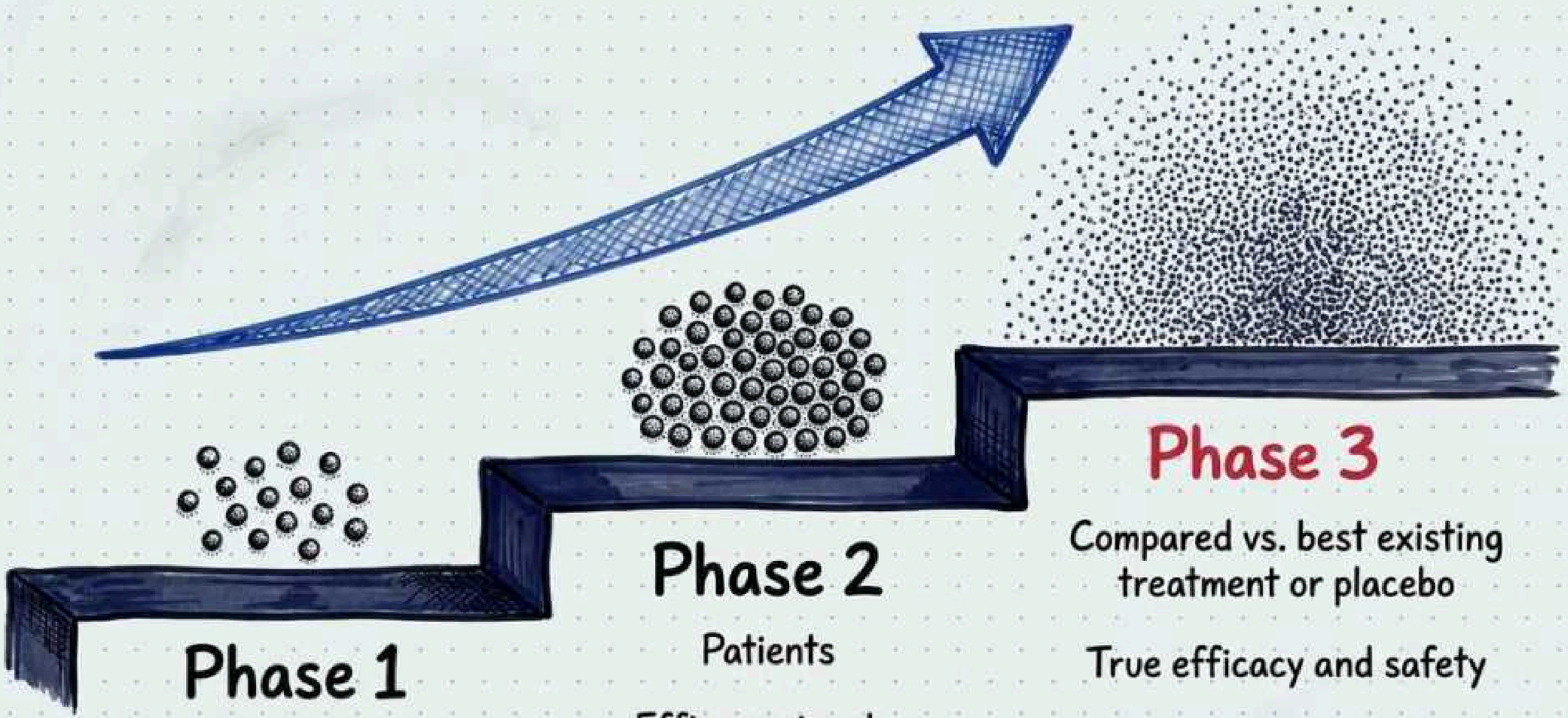


Only a small fraction judged safe/promising advance to human trials.

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Clinical Trials: Testing in People

¶6.4



Phase 1

Healthy volunteers or patients

Safety, PK handling, tolerated dose range

Phase 2

Patients

Efficacy signals, dose refinement

Phase 3

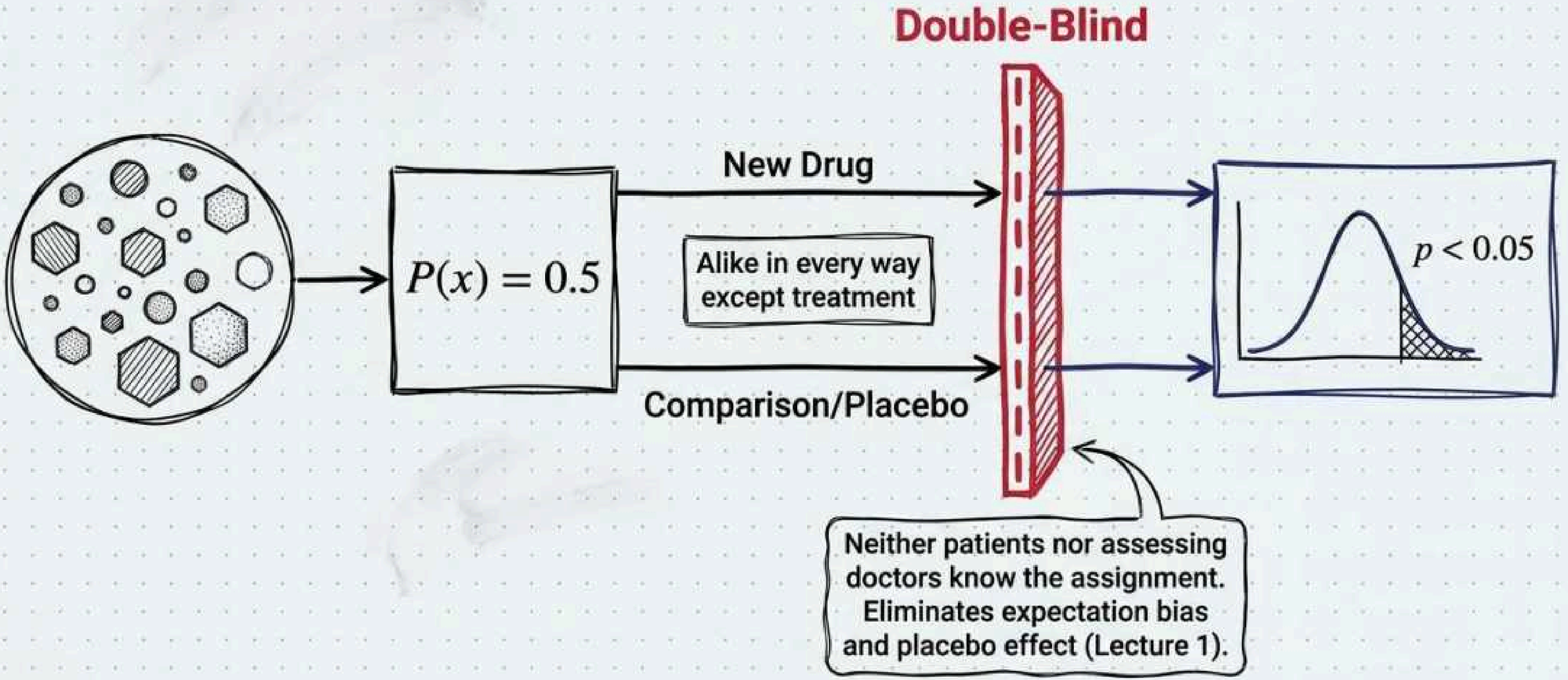
Compared vs. best existing treatment or placebo

True efficacy and safety

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Randomised Controlled Trial

¶ 6.5



¶ 6.6

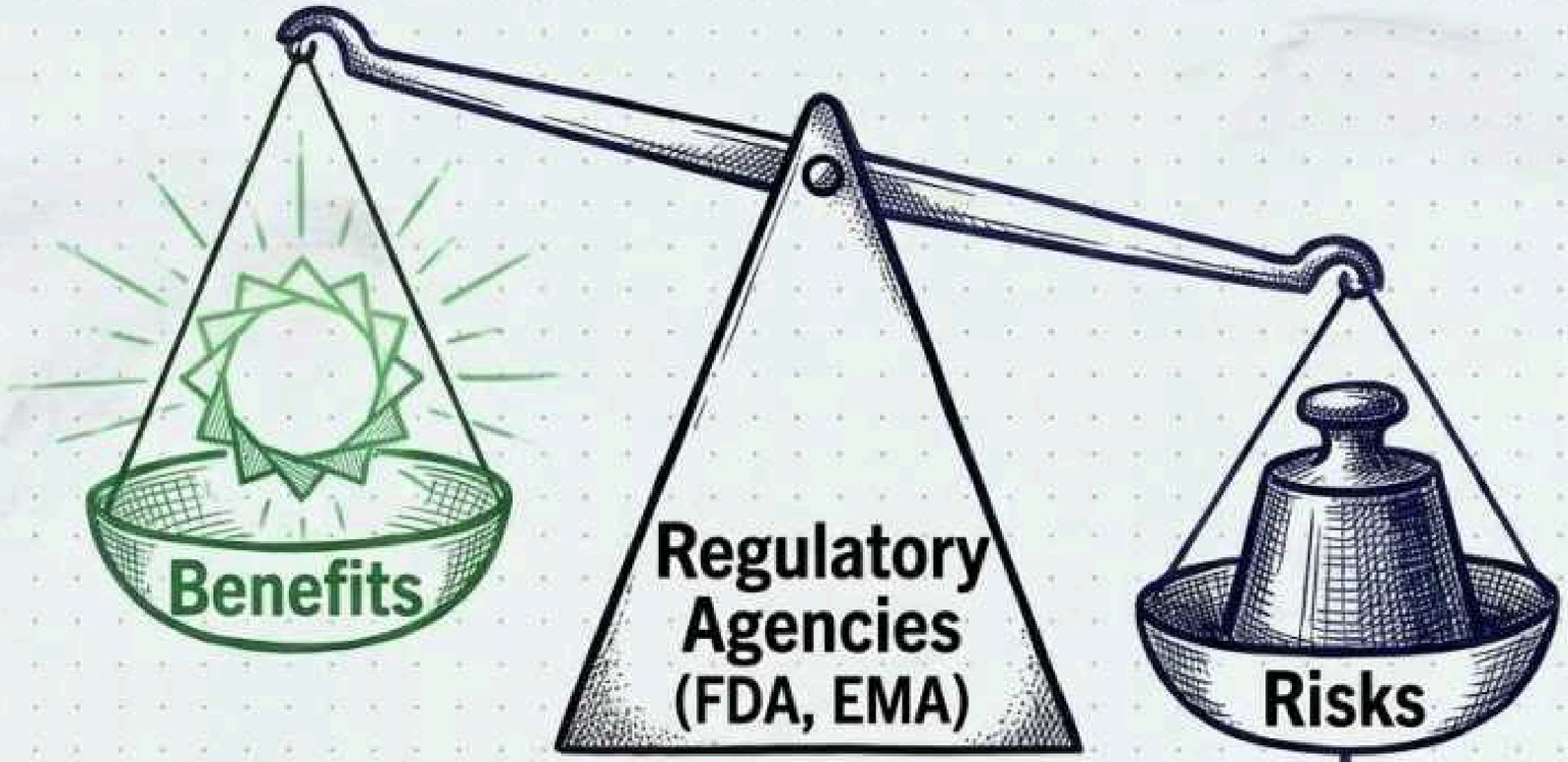
Human Clinical Trials

Connects to Bioethics: Philosophy 235 B.



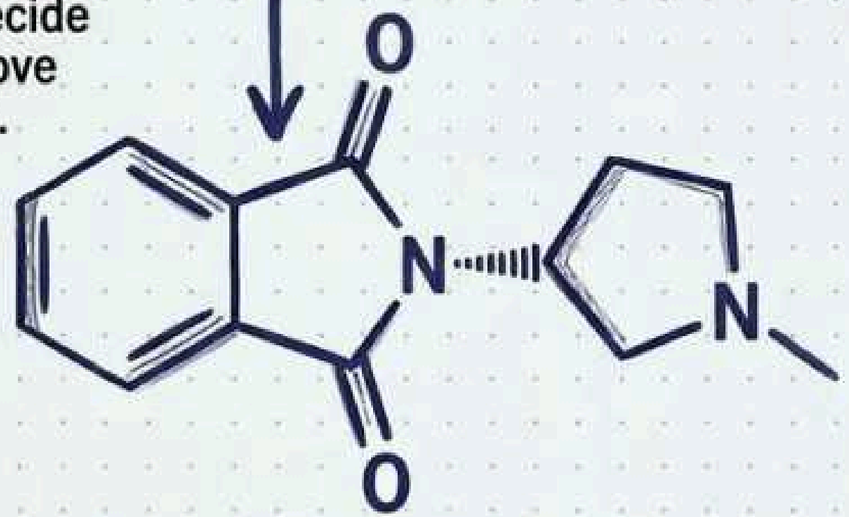
Gemini Notebook

¶ 6.7



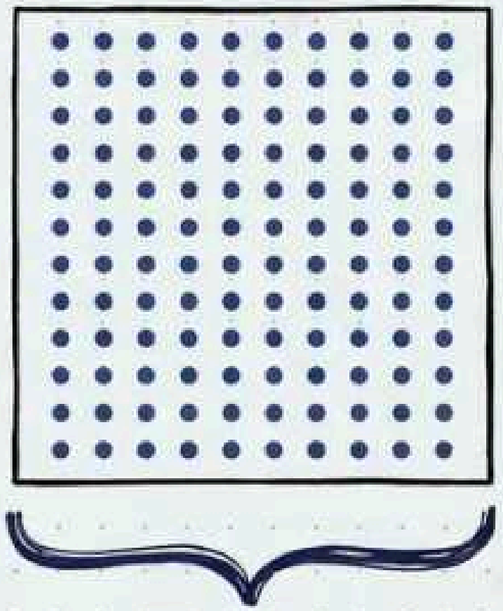
Agency experts review data, decide on a particular use, and approve accompanying information.

1960 Thalidomide Disaster:
Drug given to pregnant women caused severe birth defects. Led to massive strengthening of modern regulation.

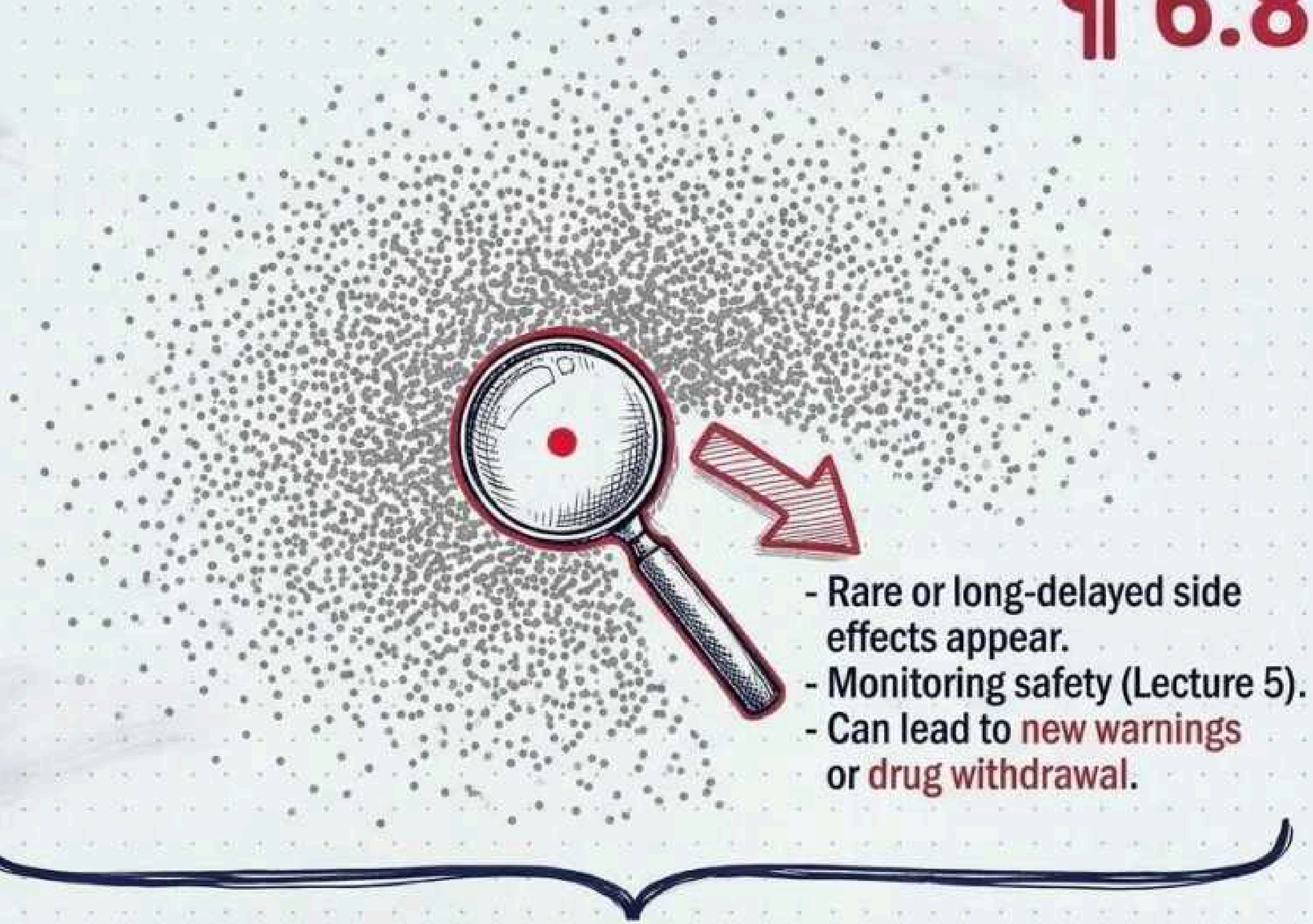


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¶ 6.8



Trials: Limited N, selected people, limited time.



- Rare or long-delayed side effects appear.
- Monitoring safety (Lecture 5).
- Can lead to new warnings or drug withdrawal.

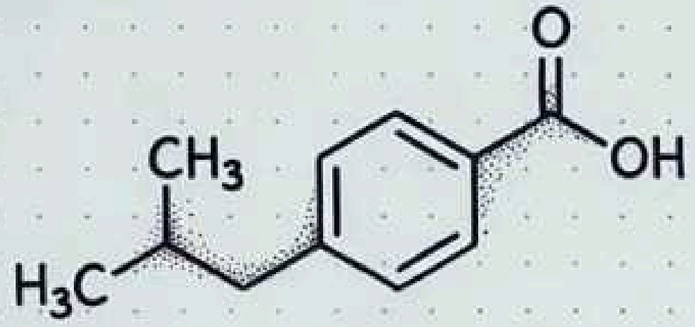
Real-World Use: Millions of people.

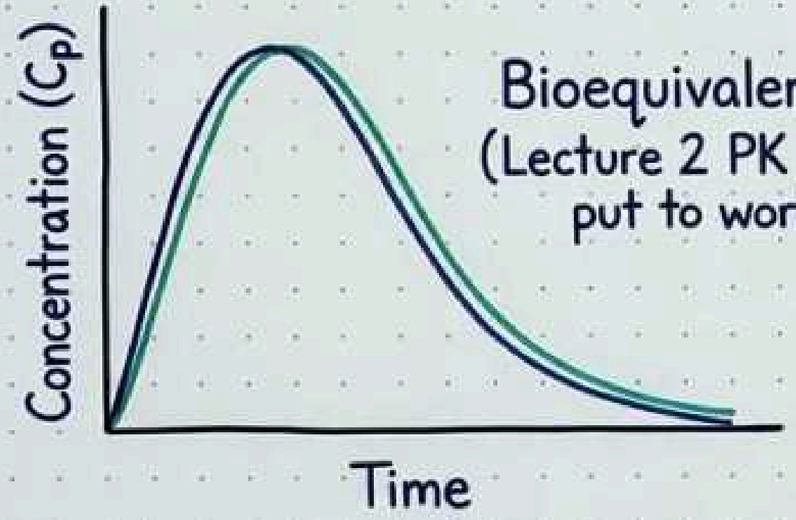
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Patent Expiration: Making Medicines Affordable

¶6.9

Generics

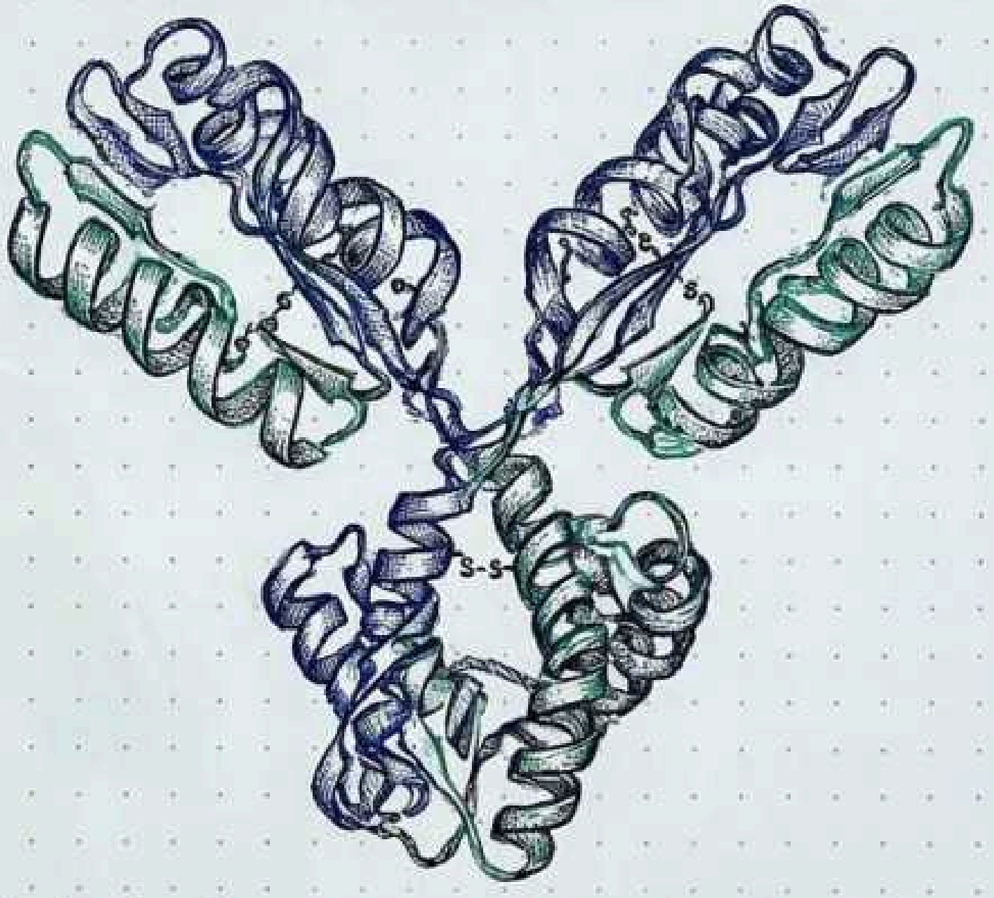




Bioequivalence
(Lecture 2 PK ideas put to work)

Same active ingredient. Delivered to body in the same way.

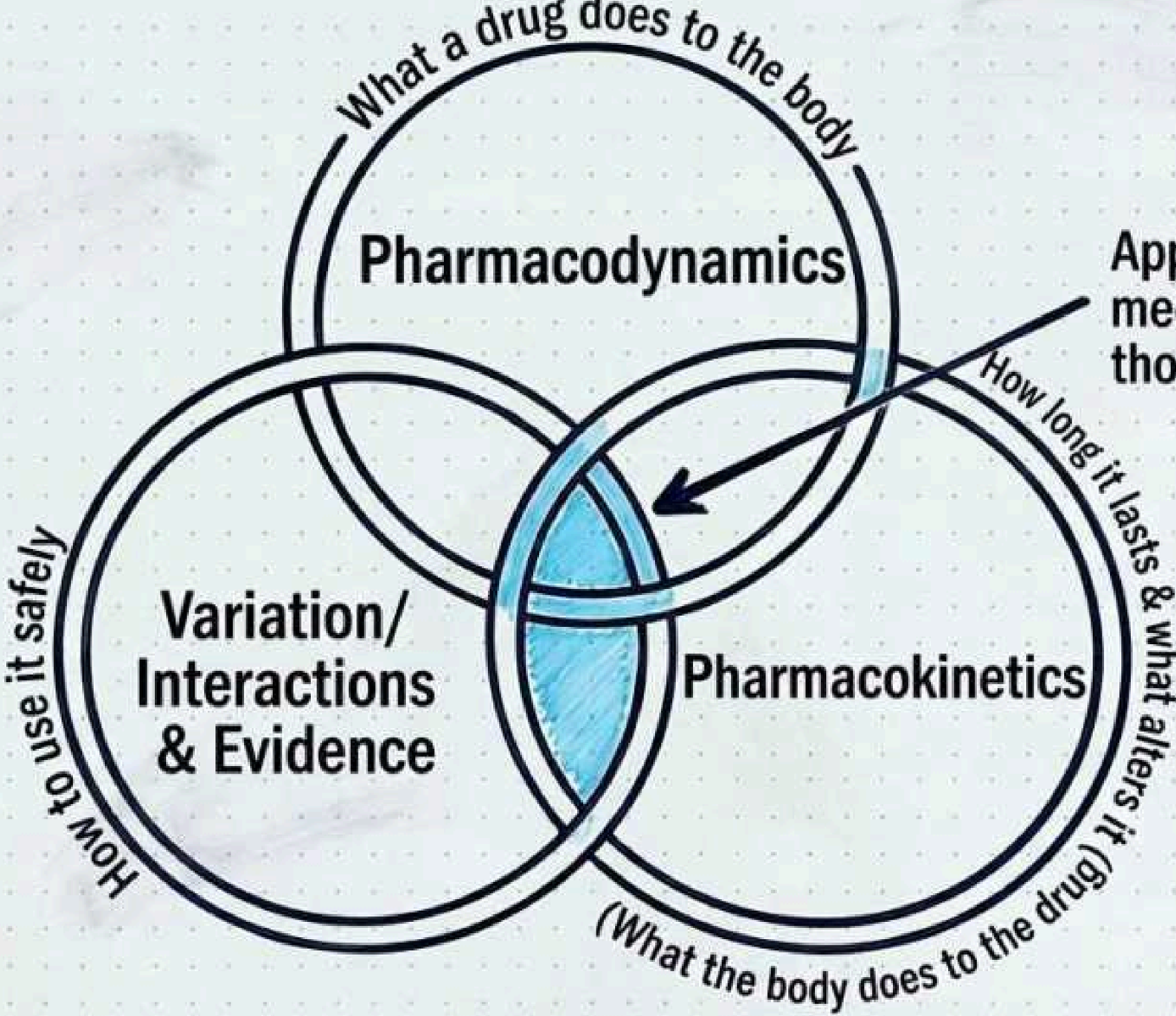
Biosimilars



Made by living cells. Large biological molecules are harder to reproduce exactly. Tested differently—similar, but not identical

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¶ 6.10



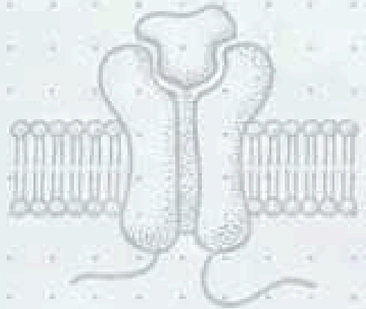
→ Next: Meeting these drugs in action, organ system by organ system.

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¶ 6.11

[Choose a Medicine]

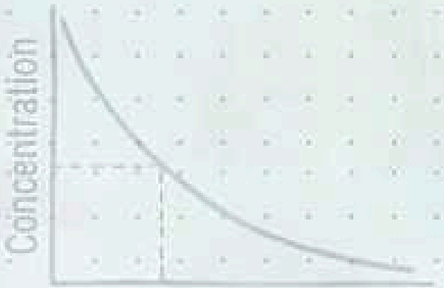
Target?



Interactions / Adverse Effects?



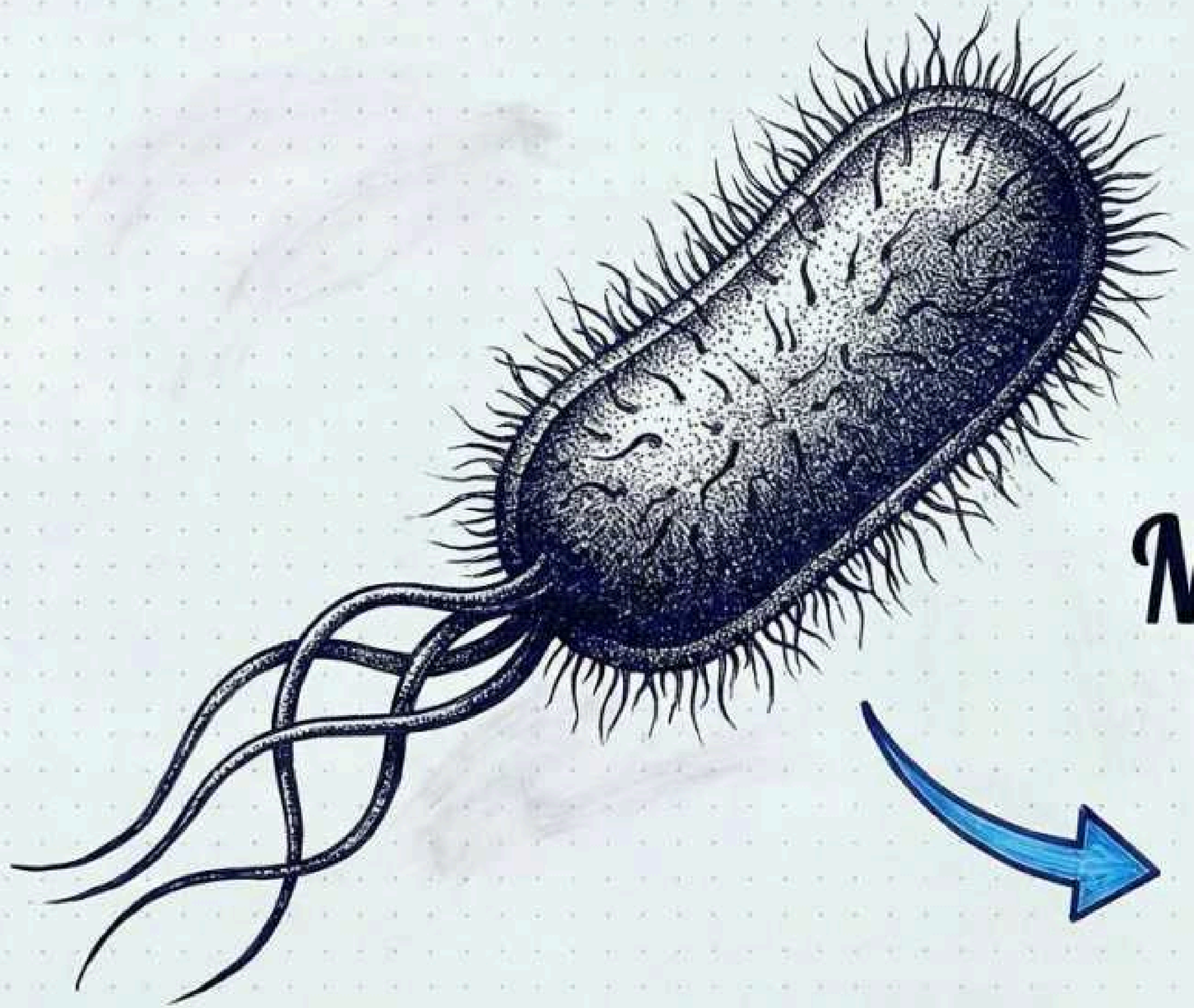
Body Handling (PK)?



Check your reasoning against the Course Book.
Remember: Real decisions belong with a doctor or pharmacist.

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¶6.12



Thank you for taking Medicine 511 B.

Next Course: Medicine 512 B – Mechanisms of Microbial Pathogenesis.

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