Last week

Drug Development & Finance

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OUTLINE

- What is a drug?
- Types of drugs
- Breakthroughs In Biomedicine

1. What is a Drug ?

What is a drug?

- Drugs are **substances** that affect the functions of living things and are administered to treat, prevent, or cure unwanted diseases and symptoms.
- The United States Food and Drug Administration (FDA) **regulates** drug marketing, requiring manufacturers to prove their products to be safe, effective, and appropriately labeled.
- Scientists start with simple, defined, model systems that enable them to identify potential drugs. These potential drugs are then tested in increasingly complex and real-world situations to prove their efficacy.

- **Enzymes** /ˈɛnzaɪmz/ are proteins that act as biological catalysts (biocatalysts). Catalysts accelerate chemical reactions.

- https://www.youtube.com/watch?v=yk14dOOvwMk

- https://www.youtube.com/watch?v=PhfhMBO-w9Q

2. Types of Drugs

PHARMACEUTICAL VS. BIOTECHNOLOGY DRUG

- Traditional pharmaceutical drugs differ from biotechnology-derived drugs in the methods by which they are **discovered and manufactured**.

- Example therapeutic **insulin**: pharmaceutical companies extracted insulin from the pancreas of pigs while Genentech produced recombinant human insulin by synthesizing it in bacteria.

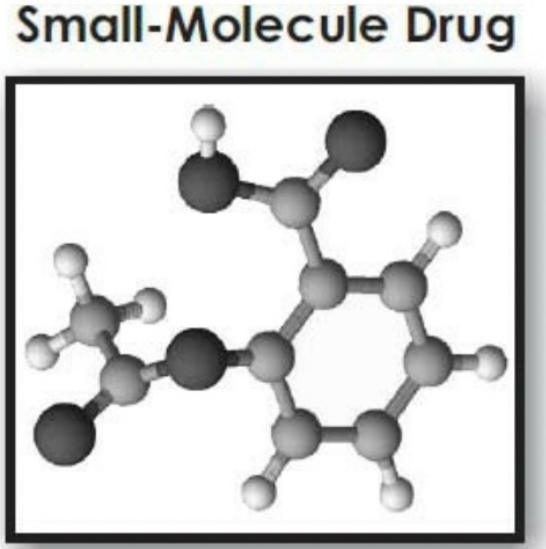
PHARMACEUTICAL VS. BIOTECHNOLOGY DRUG

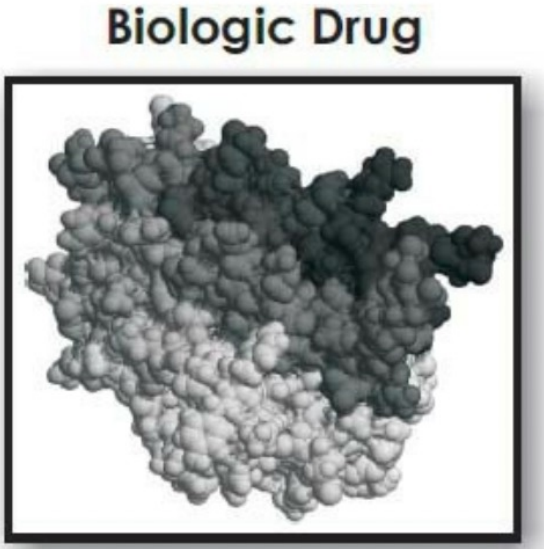
- **Traditional pharmaceutical drug** discovery was based on
- **trial-and-error** screening of synthetic compounds
- directed **selection of biological extracts** that can affect model systems.
- the emphasis of research was to understand **biological systems** in order to find potential drug targets.
- **Molecular biology techniques** used by biotechnology firms
- **directed design of biological compounds** as drug candidates.

Traditional pharmaceutical development was limited to chemical synthesis and biological extracts

PHARMACEUTICAL VS. BIOTECHNOLOGY DRUG

- **traditional pharmaceutical drugs** tend to be **small molecules** that are orally doseable as tablets, capsules, or liquids.
- **biotechnology drugs** are **proteins**, such as growth factors, monoclonal antibodies, hormones, and cytokines. Other categories include nucleic acids and vaccines.
- Biotechnology drugs are **larger and more complex** than traditional pharmaceutical drugs.
- Drug **delivery** is an issue for biotechnology-derived drugs





PHARMACEUTICAL VS. BIOTECHNOLOGY DRUG

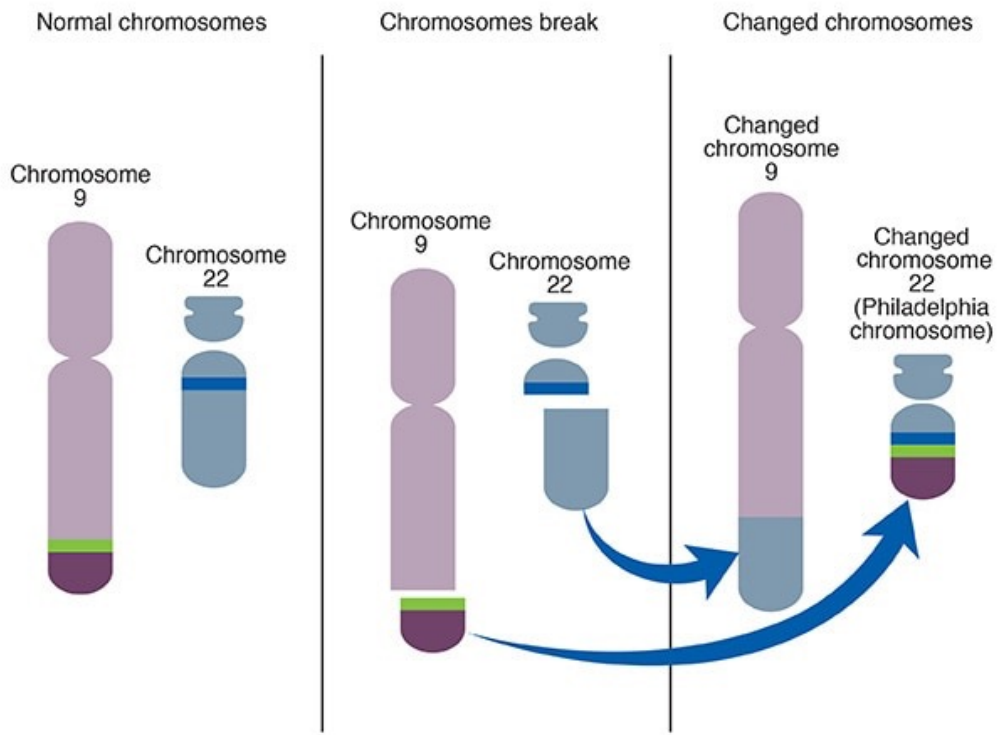
- Biosimilars vs. generic drugs
- https://www.statnews.com/2019/02/05/biosimilars-biologics-explainer-video/

3. Breakthroughs In Biomedicine

Breakthroughs In Biomedicine

- 2001: Gleevec, first of a new class of drugs based on molecular biology (tyrosine kinase inhibitor), $C_{29}H_{31}N_7O$
- 2004: Avastin, angiogenesis inhibitor (VEGF), $C_{6638}H_{10160}N_{1720}O_{2108}S_{44}$
- 2006: Sutent, approved for RCC and GIST simultaneously, $C_{22}H_{27}FN_4O_2$
- 2008: First cancer genome (leukemia) sequenced by Wash U. Genome Institute, Nature 456 (2008):66-72.
- 2012: Dr. Lukas Wartman, Wash U. “cured” of acute lymphoblastic leukemia via RNA analysis and Sutent
- 2012: David Aponte “cured” of same type of leukemia using immunotherapy (T-cells targeting CD19)
- 2017: Luxturna gene therapy approved for treating Leber’s congenital amaurosis

Chronic myelogenous leukemia



How Gleevec work

- Is an inhibitor of enzymes
- Does not change the gene
- https://dnalc.cshl.edu/resources/3d/32-how-gleevec-works.html

Biomedicine Is At An Inflection Point

Spark THERAPEUTICS

Dec 19, 2017

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FDA Approves Spark Therapeutics' LUXTURNA™ (voretigene neparvovec-rzyl), a One-time Gene Therapy for Patients with Confirmed Biallelic RPE65 Mutation-associated Retinal Dystrophy

"I went outside when it was snowing, and I was like, 'Oh! I can see the snowflakes!'…It was really cool to actually see something that I've never seen in my life before."