Intro to Pharmaceutical Research

What is Pharmacology?

Pharmacology: branch of science concerned with drug or medical action

Drug: Substance that causes a pathophysiological response.

Types of Classifications:

1. Therapeutic
2. Pharmacological

Naming Convention of Drugs

1. Chemical name
2. Generic name
3. Brand name

Pharmacokinetics: How a drug moves through the body

Pharmacodynamics: How drug affects the body

Cancer

What is Cancer?

Cancer is a disease in which healthy body cells mutate and multiply rapidly. Tumors can be cancerous or benign. They can also spread from one area in the body to another through a process called metastasis. Cancer is both genetic, a result of the environment, and chance.

Different body cells have different functions but share a similar basic structure. This is part of why cancer is prevalent in all parts of the body.

Gene mutation may result in too many copies of a chromosome, a missing chromosome, or a misplaced chromosome. These mutations mean that

- A cell starts making too many proteins that triggers a cell to divide
- A cell stops making proteins that tell a cell to stop dividing
- Abnormal proteins work differently than usual

Current Frontiers

Cancer is the biggest medical challenge in the world. 1 in 2 adults in the US will experience some form of cancer in their lifetime.

Some research that can be carried out

- Population based research
  - Using datasets and statistics to analyze trends and common factors
- Health services research
  - Focuses on the quality, accessibility, and cost of healthcare
- Treatments and therapies
  - Nanoparticles
  - Genetically-Engineered Whole-Cell Vaccines
  - Gene Editing
  - Drugs

Allergy and Infectious Diseases

What are infectious diseases?

Infectious diseases are caused by organisms like **viruses and bacteria**, and are usually contagious.

- COVID-19
- Flu
- Common Cold
- Ebola

Treatment for an infectious disease is dependent on the **cause** of the disease

- Bacteria -> Antibiotics
- Viral -> Treated with supportive therapies, prevented by vaccines
- Fungal/Parasitic -> Treated with antifungals (Diflucan) and antiparasitics (EmverM)

Outcomes will vary based on the cause of the disease, some patients will recover fully, many will not.

Current Frontiers

Infectious disease research is one of the fastest growing fields in medicinal research. There is an abundance of data that currently exists that can be worked with. For any infectious disease, there are 3 main areas that you can research:

- Means and patterns w/ disease transmission
  - Using open source datasets and performing statistical analysis.
  - Finding at-risk groups of people
- Infectious pathway and effect on the host.
  - Finding how a pathogen binds and enters a cell
  - Spread of pathogen from cell to cell
- Treatments
  - Vaccines
  - Therapies
  - Antibiotics
  - Antifungals

Heart, Lung, and Blood

Cardiology, Pulmonology, and Hematology

Cardiology: The branch of medicine encompassing anything and everything that has to do with your heart

Pulmonology: The branch of medicine focusing on diseases that impact your respiratory tract

Hematology: The three step process (prognosis, treatment, prevention) of blood related diseases

Primary Issues within each specialty

Cardiology	Pulmonology	Hematology
Diseases: Blood vessel disease, Coronary artery disease, Arrhythmias, Heart Valve Disease	**Diseases**: Pneumothorax, Pneumonia, COPD, Bronchitis, Emphysema	**Diseases**: Anemia, Sickle Cell Diseases, Hemophilia, Leukocytosis

Treatment

Cardiology	Pulmonology	Hematology
Angiotensin-2 receptor blockers (ARBs): blocks the action of angiotensin to help lower blood pressure **Nitroglycerin**: Relaxes blood vessels to further blood flow, relieves pressure on heart	**Bronchodilators**: drugs that relieve coughing and breathing problems by relaxing constricted airways **Infusions of AAT to help slow the progression of lung damage**: helps with alpha-1 antitrypsin (AAT) deficiency	**Car T-cell therapy**: uses a patient's own immune system for targeted attacks on cancer **Stem Cell Transplantation**: chemotherapy is used to kill infected cells and then transplanted stem cells are inserted

General Medical Sciences

What is General Medical Sciences?

Basic research exploring biological processes and living systems - behavior of molecules in cells, tissues and organs in living research model organisms. Lays the groundwork for work with diseases

Broader areas, not specific - multiple organ systems:

Anesthesiology & perioperative pain

Sepsis

Non-specific clinical pharmacology

Trauma, burn injuries, wound healing

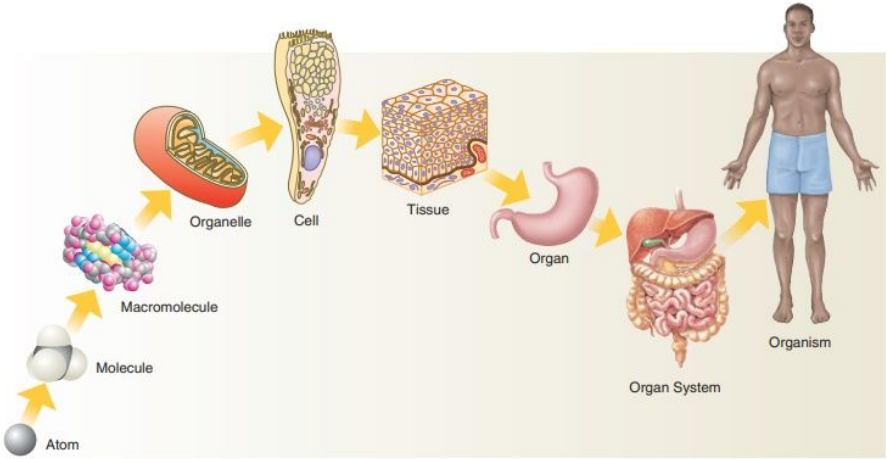

FIGURE 1-1 Organization levels of the body.

Adapted from Shier, D.N., Butler, J.L., and Lewis, R. Hole's Essentials of Human Anatomy & Physiology, Tenth edition. McGraw Hill Higher Education, 2009.

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Current Frontiers

There are a lot of possibilities in the field of GMS; any discovery may ultimately have many applications.

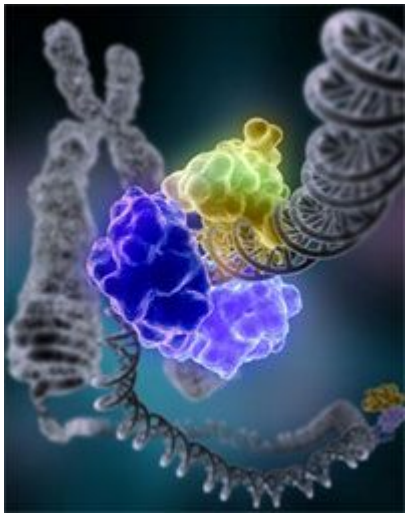
Research ideas:

Design custom proteins to serve specialized functions

Use cell imaging to investigate intracellular and extracellular processes and mechanisms

- Gene regulation
- Cell cycle regulation
- Cell signaling
- etc.

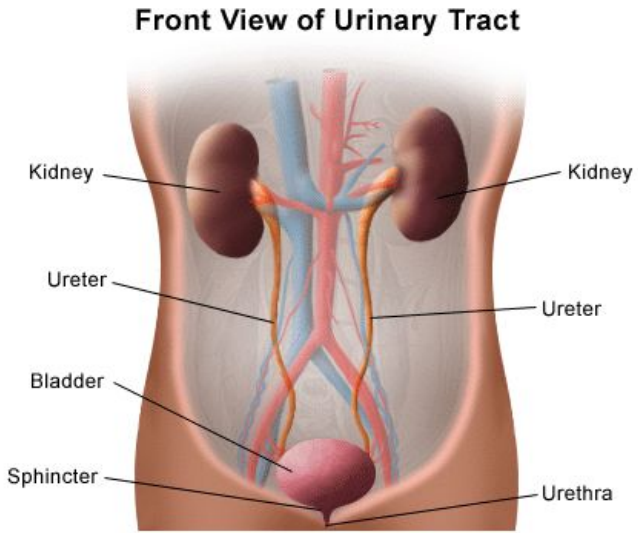
Model disease outbreaks



Diabetes and Digestive and Kidney Diseases

Diabetes, Urology, Digestive System

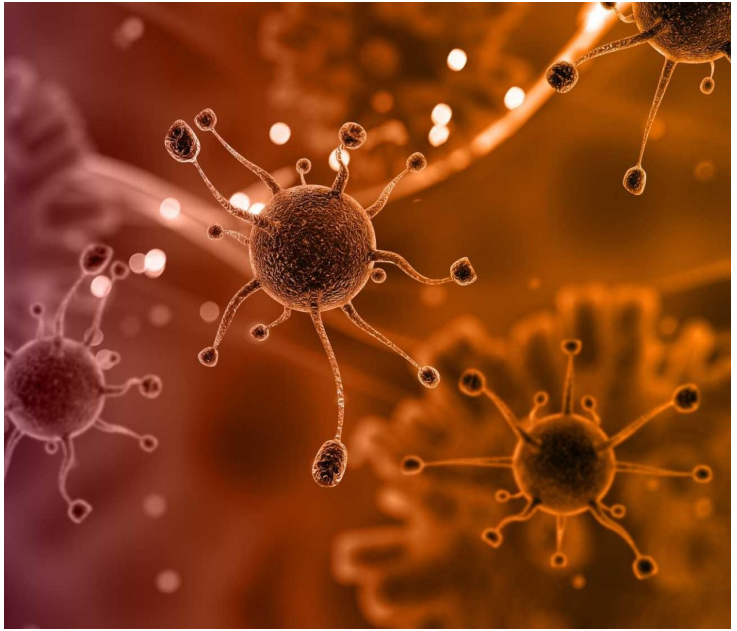
- **Diabetes:**
  - Diseases that result in high blood glucose levels
- **Urinary System:**
  - Eliminate waste
  - Regulate blood volume/pressure/pH
  - Control levels of electrolytes/metabolites
- **Digestive System:**
  - Breaks down foods into nutrients
  - Absorbed into bloodstream for energy, growth, and repair



Current Frontiers

Diseases: Type 1/2 Diabetes, Crohns, Celiac, Cystitis, Cancer

- Current Research:
  - **Diabetes**: Low insulin levels→Regeneration of beta cells in diabetes
  - **Crohn's:** Ustekinumab binds to both IL-12 and IL-23 which prevents them from working.
  - **Kidney Cancer:** Mitomycin C works by interfering with the development of the genetic material in a cell, the DNA. (stopping cycle)



Neurological Disorders

The Brain and the Nervous System

Neurological/nervous system disorders are defined as disorders that originate from and affect the brain as well as nerves found throughout the human body and the spinal cord.

Structural, biochemical, or electrical abnormalities in the brain and the peripheral and central nervous system can result in a range of symptoms and can have a wide range of causes.

Translational research and clinical trials are utilized to further research in this area.

List of Disorders

- Acute spinal cord injury
- Alzheimer’s disease
- Amyotrophic Lateral Sclerosis (ALS)
- Ataxia
- Bell's Palsy
- Cerebral Aneurysm
- Epilepsy and Seizures
- Guillain-Barré Syndrome
- Head injury
- Hydrocephalus
- Lumbar disk disease
- Meningitis
- Multiple sclerosis
- Muscular dystrophy
- Parkinson's disease
- Stroke

Specific Research Interests

Stroke

Movement disorders

Brain imaging

Neurogenetics

Neuroimmunology

Surgical neurology

Neurovirology

Integrative neuroscience

Molecular biophysics

Synapses and circuits

Neuronal development

Mental Health

What is Mental Health?

- Mental Health: A person's emotional, mental, and psychological well being (CDC)
  - relationships, ability to cope with adversity, carry out productive activities, etc.
- Mental illnesses are health conditions
  - changes in emotion, thinking or behavior (or a combination of these).
  - Distress and/or problems functioning in social, work or family activities. (American Psychiatric Association)
  - Psychosis: Condition so severe that patient has lost all touch with reality
- Statistics
  - >50% of Americans will experience mental illness/disorder at some point
  - 1 in 25 Americans lives with a serious mental illness (schizophrenia, bipolar disorder, or major depression)

Potential Research Avenues

- Impact of genes/epigenomic regulators on the severity of mental illnesses
  - expression quantitative trait loci govern multiple genes' expression (LINK)
    - SETD1A on Schizophrenia (LINK)
  - Changes in epigenetic markers as a result of stress, environment, etc.
- Identify and validate new molecular targets and tools for drug discovery relevant to the treatment of mental disorders.
- Deep Brain Stimulation as treatment for Treatment Resistant Depression (LINK)
- Elucidate fundamental mechanisms (e.g., genetic, biological, behavioral, environmental) of complex social behavior. (LINK)
- EPINET(Early Psychosis Intervention Network)

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