

Course Title: Drugs and Disease – Spring 2025

Course Materials: https://www.andrew.cmu.edu/user/rule/Drugs_Disease/

Professor: Gordon Rule. Professor of Biochemistry (rule@andrew.cmu.edu)

Teaching Assistant: Ying Zhai <yzha869@aucklanduni.ac.nz>

Course Description:

This course will discuss the action of selected treatments of disease, including genetic deficiencies as well as diseases caused by pathogens.

The Topic in Global Context:

Advances in drugs and other therapies has led to the successful management of many diseases world-wide.

Required Readings:

Prior to beginning this course, it would be helpful if students reviewed high-school chemistry, in particular topics in organic chemistry. The following readings from the free online biology textbook:

<https://openstax.org/details/books/biology-2e>

Chapter 2 – The Chemical Foundation of Life

Chapter 14 – DNA structure and Function

Chapter 3 – Biological Macromolecules

Chapter 15 – Genes and proteins.

Chapter 4 – Cell Structure

Assignment:

- Students will be expected to complete a short problem set after each lecture. The problem sets are designed to increase the depth and understanding of the lecture material.
- Students will also be expected to give a final presentation and prepare a final paper based on the course material. The topic of the presentation and the paper are chosen by the student (see below).

The student paper and presentation should have the following structure:

- i) biological background on the disease, explained at the level of a first-year university student.
- ii) how the methodology treats the disease (e.g. what process does the drug interfere with),
- iii) positives and negatives of this treatment and a comparison to other treatments (if applicable).

The paper should be no more than **2 pages** in length (11 pt font, 1 cm margins, single spaced). Figures should not occupy more than 25% of this space. All external sources should be cited properly. There is no page limit on the citations.

The presentation should be a professional powerpoint presentation. Each presentation should be **7 min**.

Please respect the following due dates (see course schedule):

Feb 3 @ 11:59 PM EDT – Presentation topic for approval, email to rule@andrew.cmu.edu

Feb 10 @ 11:59 PM EDT – Draft slides for feedback, email to rule@andrew.cmu.edu

Feb 15 – Presentation (normal class meeting time)

Feb 22 @ 11:59 PM EDT – Final presentation and paper due, email to rule@andrew.cmu.edu

Suggested list of the topics for the final project:

The student can pick any topic that is related to drug action or the treatment of disease by antibody therapeutics or genome editing. Although students can select a topic that is also covered in lecture, they are encouraged to select topics that are not directly covered in lecture. If a topic is selected that was covered in lecture, then the expectation will be for the student to go deeper into that topic or discuss a different aspect of the topic.

Assessment:

60% - Problem sets (10% each problem set).

25% - Final Paper

15% - Final Presentation

Class Expectation & a word for the students:

This course is designed to give you the fundamental skills to understand current therapies for the treatment of diseases. The final presentation and paper will give you an opportunity to showcase those skills.

Please ask questions during lecture and please complete the homework after each class. If you have questions, please feel free to email them to me and I will answer promptly.

Overview of Course:

Date	#	Topics (Tentative Schedule, some topics may carry over)	Due Dates
Jan 15	1	Objective: Develop an understanding of the fundamentals of Chemistry, Protein structure and function. <ul style="list-style-type: none"> • Chemical bonding • Protein structure and function <ul style="list-style-type: none"> ○ Protein Folding diseases ○ Ligand binding 	Problem Set 1: Based on assigned pre-course readings. Completed problem sets should be emailed to the instructor and the course TA. <i>They are due the day of the class, @ 11:59 EST.</i>
Jan 24	2	Objective: Develop an understanding of fundamentals of Basic enzymology, carbohydrates, and lipids. Description: <ul style="list-style-type: none"> • Basic enzymology • Introduction to carbohydrates • Lipids and membranes (Cholesterol regulation) 	Problem Set 2: Based on lec. 1.
		Pre-lecture video on basic nucleic acid structure.	
Jan 25	3	Object: Develop an understanding of fundamentals of Nucleic acid Structure and function, Central Dogma of Biology Description: <ul style="list-style-type: none"> • Nucleic acid structure (Pre lecture video) • Central Dogma of Biology (Transcription & Translation) • Biotechnology – DNA sequencing & PCR 	Problem Set 3: Based on lec 2.
Feb 5	4	Objective: Understand process of acquired immunity and the production of antibodies as part of the immune response. Explore the use of antibodies as drugs. Description: <ul style="list-style-type: none"> • Overview of acquired immunity • Antibody diversity and production • Antibody structural components • Antibodies in cancer treatment • Antibodies in drug detoxification 	Problem Set 4: Based on lec 3.
		Pre-lecture video on basic enzyme kinetics	Presentation Topic Due
Feb 8	5	Objective A: Development and characterization of enzyme inhibitors as drugs. Description: <ul style="list-style-type: none"> • Enzyme inhibitors (suicide, competitive, allosteric) • Antivirals for the treatment of HIV • Statins for the treatment of hyper-cholesterol Objective B: Understanding methods to correct genetic diseases by gene editing. Description: <ul style="list-style-type: none"> • Overview of selected genetic diseases: <ul style="list-style-type: none"> ○ Immunodeficiencies • Gene editing using viral vectors • Gene editing using CRISPR-Cas9 	Problem Set 5: Based on lec 4 & 5.
Feb 10			Presentation Slides Due (Draft).
Feb 15		Final Presentations	
Feb 22			Final presentation slides. Final report