**Course Information**

Meetings: 9:30 AM–10:45 AM Tuesdays & Thursdays, Gilmer 141  
Instructor: Dr. Michael Wormington  
Office: PLSB 206  
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Office Hours: 2:00 – 4:00 PM Tuesdays, PLSB 200 & by appointment  
Prerequisite: BIOL 3000

**Course Objectives**

The overall goal of BIOL 4260 is for you to gain experience in critically reading and assessing the primary scientific literature and to learn to think like an experimental molecular cell biologist addressing “translational research”. By the end of this course you will be able to:

1. Critically read and assess, interpret, and present the data and conclusions posed in primary research articles.
2. Describe and explain the pertinent molecular and cellular processes that underlie the specific diseases under discussion.
3. Describe and explain how specific genes and/or their mRNA or protein products can be validated as therapeutic targets to develop new treatment options and approaches.
4. Identify the strengths and weaknesses of different drug discovery approaches utilized to develop new therapeutic options for genetic diseases.
5. Develop the ability to give concise, lucid, and well-organized oral presentations and to work collaborative in a “team setting”.

**Course Description**

This course will use a case study approach to examine diverse genetic diseases including, but not limited to, ALS, Cancer, Cystic Fibrosis, Diabetes, Duchenne Muscular Dystrophy, and Huntington-Gilford Progeria. Additional diseases will be considered based on student interests and recent research advances. The overall goal is to examine specific regulatory or mechanistic events that underlie the diseased state and their associated molecular components that may serve as new therapeutic targets. We will explore novel treatment options under development and consider their relative strengths and weaknesses. These approaches range from small molecules that function variously as chaperones, nonsense suppressors or RNA splicing modulators to induced pluripotent stem cells and gene repair. We will also discuss new findings that elucidate the basis for diseases for which there are currently no treatments and consider how novel therapeutic options could be developed. Assigned reading will come from the primary scientific literature. As such, a major objective of this course will be to provide an opportunity, to learn how to critically read, interpret, and evaluate primary research papers. Although no textbook will be used, relevant background material can be found in the Lodish et al Molecular Cell Biology text used in BIOL 3000.

**Grading**

This is a discussion-intensive class. Therefore, class attendance and active participation are essential. You will be working in small groups to discuss the assigned papers using a set of specific questions provided beforehand as guidelines. Each group will then be tasked with summarizing their discussions of various questions with the entire class. 60% of your course grade will be based on class participation. 20% of your course grade will be based on short take-home "open book" exercises associated with each topic. 20% of your course grade will be based on a final group project in which you will have the opportunity to identify and validate a molecular target, and develop a therapeutic approach for a disease of your choice. Details on presentation length & format will be provided once the course is under way. You will be explicitly told which assignments can be completed as a group effort & those that must be completed individually.