**BI 23. Current Advances in Gene Therapies**

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Syllabus

Gene therapy holds the promise of finding cures to some of the most problematic diseases. Unlike the commonly used small molecule drugs, genes expressed in the body can perform more complex tasks and be better targeted to disease sites. In this course we will cover topics ranging from the need for gene therapy, through biochemical and device-based the methods of gene delivery, to challenges facing current therapies. We will finish the course with case studies of successful gene therapies that are already on the market, and examine why gene therapies of the previous decades have failed.

1) The need for gene therapy – difference between gene therapies and small molecule drugs.

2) Methods of gene delivery I: non-viral approaches.

3) Methods of gene delivery II: viral approaches.

4) Methods of gene delivery III: gene delivery to the brain.

5) Immunogenicity in gene therapy I – viral vectors.

6) Immunogenicity in gene therapy II: transgene immunogenicity.

**- Midterm paper: due date (Due Feb. 6th); News/perspective piece on the recently developed gene delivery vector/method.**

7) Gene editing.

8) Applications of gene therapy in scientific research.

9) Applications of gene therapy in hemophilia, blindness, and other disorders.

10) Future challenges and promise of upcoming therapies.

**- Final paper (Due March 20th): Your concept of a gene therapy: choose a vector, transgene, and a method of gene delivery for a currently untreatable disease, explain the intended mechanism of action.**

**Meeting times:** We should meet once per week and the hours will be flexible. I do have some talks to give out of town next quarter but I should know the dates at least a month ahead of time.

**Grading:** This is a Pass-Fail course. Grade will be based on attendance (30%), midterm paper (1-2 pages, 30%), and the final paper (2-3 pages, 40%). Evaluation of the papers will be based on the factual correctness of the statement, completeness, citing sources, more than the quality of writing. The midterm should include one figure, and the final paper at least two. You will receive the feedback on the assignments.

**Assigned reading**: There will be a total of 5-10 review papers to read through in total. These should be relatively easy reads that give a big-picture overview of the field. Since term papers deal with specific subjects, you may need to read a few research studies. The goal is to average out 1.5-2 h of work per week on a normal week, and spend ca 6 h for midterm and 10 h on the final paper, for a total workload of ~30 h in the quarter.

**Learning goals**: I think it would be useful if at the end of the course you could identify strengths and weaknesses of gene therapeutics in comparison to biologics and small molecules. Additionally I am hoping you will be able to read a review, or a news&views article and approximate how feasible will the approach be in your research, or in the clinic. Finally, I hope that you will find this topic exciting and it will push you to learn more on your own. With all that said, I am waiting for your feedback to hear what you want to get out of the class.