

MVIMG (Molecular Virology, Immunology and Medical Genetics)#: 7470
Neuromuscular Biology and Disease, Spring Semester 2014

Duchenne Muscular Dystrophy

Course Instructors: Denis Guttridge and Jill Rafael-Fortney

Thursdays 2:30PM-3:30PM, Parks Hall 103

Course Outline (Fourteen 55 minute lectures or discussions)

1. Jan 9

Clinical/genetics/diagnosis overview - Kevin Flanigan, M.D.

History of dystrophin gene cloning - Arthur Burghes, Ph.D.

2. Jan 16

Overview of skeletal and cardiac muscle physiology and measurements - Paul Janssen, Ph.D.

3. Jan 23

Histopathology/ pathophysiology overview - Zarife Sahenk, M.D., Ph.D. / Jill Rafael-Fortney, Ph.D.

4. Jan 30

Dystrophin glycoprotein complex discovery and associations - Rachele Crosbie, Ph.D. (UCLA)

5. Feb 6

Cardiomyopathy - Subha Raman, M.D.

6. Feb 13

Discussion of Darius Goes West - Denis Guttridge, Ph.D. and Jill Rafael-Fortney, Ph.D.

Required preparation:

- Darius Goes West - out of class assignment

To watch film on-line go to: <http://www.dariusgoeswest.org/playitforward/>

7. Feb 20

Summary of preclinical and transgenic models - Jill Rafael-Fortney, Ph.D.

8. Feb 27

Membrane damage and repair - Noah Weisleder, Ph.D.

9. Mar 6

Glycosylation and membrane stabilization - Paul Martin, Ph.D.

Spring Break

10. Mar 20

Inflammation - Jim Tidball, Ph.D. (UCLA)

11. Mar 27

Signaling pathways - Denis Guttridge, Ph.D.

12. Apr 3

Fibrosis - Federica Montanaro, Ph.D.

13. Apr 10

Gene therapy/ exon skipping/ pre/neonatal-testing - Jerry Mendell, M.D.

14. Apr 17

Perspective from a parent and advocate - Pat Furlong, Parent Project Muscular Dystrophy

Course Materials

Suggested reading to support each lecture or discussion will be posted on Carmen one week before each lecture. Students are encouraged to read at least the first reading material listed for each class to support the information being presented. Watching the movie "Darius Goes West" is required prior to class on February 13th. The film is available free on-line at: <http://www.dariusgoeswest.org/playitforward/>

Method of Evaluation

Attendance will be recorded for each class and 10% of the overall grade will be based on attendance and participation. One paper due on the last day of class (April 17), consisting of a research proposal, will count for 70% of the overall grade. In order to assist students with learning the writing process for a research proposal, an outline with specified subheadings will be due at 9 AM on Monday, April 7th uploaded in Carmen Dropbox (under Activities), and will count for 20% of the overall grade. The outline will be edited by the instructors within a few days and returned to each student for use in preparing their final paper. The outline format will be distributed in class on February 13th and a short discussion of expectations for the outline and proposal will occur during this class period.

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