Principles and Practices of Gene Therapy (XGEN201)

Course Syllabus

Course Description
Gene therapy is promising to be key in the battle against cancer, inherited disorders, and many other diseases. Decades worth of advances in this field have resulted in a growing number of successful clinical trials to develop safe and effective treatments. Over the past few years scientists have developed a number of new nucleic acid-based therapies, which continue to improve the versatility of these genetic-based treatment approaches. In this course, you will start by building a fundamental understanding of gene therapy, then dive deeper with an in-depth look at important trends, research and advances in gene therapy. You will gain a clear understanding of how gene therapy works, how it has developed and advanced, and how much potential it has.

This course is an elective course in the Stanford Genetics and Genomics Certificate.

Course Topics
Module 1: Class Introduction
Mark A. Kay

Module 2: Fundamentals of Gene Therapeutics
Mark A. Kay
Module Exercises-
- Reading
- Activity: Understanding Vectors Used in Gene Therapeutics

Module 3: Bench to Bedside
Mark A. Kay

Module 4: Clinical Trials for Primary Immunodeficiencies and Other Diseases
Maria Grazia Roncarolo

Module 5: Genome Editing
Adi Barzel
Module Exercises-
- Reading
- Activity: Understanding Vectors Used in Gene Therapeutics 2

Module 6: Transcriptional RNAi and Other Non-Coding RNAs
Mark A. Kay

Module 7: Gene Therapy in Practice
Mark A. Kay
Instructors
Mark A. Kay
Professor of Pediatrics and of Genetics, Stanford University

Maria Grazia Roncarolo
Professor of Pediatrics and of Medicine, Stanford University

Adi Barzel
Lecturer, Stanford University

Course Requirements
Please watch all course videos and complete all course assignments. Successful completion of the assignments, final examination and course evaluation are required to complete this course. The exam consists of multiple choice questions and is done online. A link will unlock within the “Final Steps” section of the learning platform after you have completed all of the other course activities.

You may attempt the final examination multiple times. A score of 90% is required to successfully pass the exam. Once you have passed the examination and completed the evaluation, a digital record of completion will be emailed to you.

Exercises
Each exercise will be submitted via the course assignment submission area within the course learning platform. To successfully complete each exercise, you will need to follow all instructions. You will be receiving instructor feedback on some of your submitted assignments. Feedback on those exercises will be given within the learning platform within 3-5 business days. Please continue to progress through the course while awaiting instructor feedback.

Your responses to the exercises will be kept within the learning platform. However, you will not have access to the responses you submitted after 90-day course access has expired. If you would like to keep a copy for your records, please do so separately.

Please note that some assignments may contain Macromedia Flash movies or Java applets. Notes about these requirements will be included in the assignment instructions.

Course Materials
All course materials are provided within the course learning platform. These include the course videos, course handouts and all assignment instructions.

The course learning platform is available to you for 90-days after date of enrollment via your mystanfordconnection account. For more information regarding how to use the course learning platform, please visit http://player.vimeo.com/external/99190590.hd.mp4?s=02b5cdd84bc1d9e48f2320ce1d15b25b

Questions
Please contact SCPD Student Services at stanford-genetics@stanford.edu or 650-263-4700. Available 8:30am- 4:30pm Pacific Time, Monday- Friday.