Introduction:

In 2011, the FDA approved 35 new drugs--one of the highest approval volumes in the past decade. Drug development is a long and costly undertaking that involves integrated efforts from academia, industry, hospitals, and government agencies. This seminar will explain the process of developing new drugs and will focus on the concepts behind novel drug design. Technologies like high-throughput screening and 3-D protein modeling inform the design of small molecule-based drugs. Recombinant DNA technology has made it possible to make protein-based drugs. Emerging therapeutics like gene therapy and RNA interference hold possibilities for new treatment options. The biology behind drugs and examples of drug design will be covered.

Speakers:

Vini Mani is a first year PhD student in the Immunology Department. She obtained her bachelor’s degree in Bioengineering from University of California, Berkeley in May 2012. Her past experience involves working at pharmaceutical and biotechnology companies, in both R&D and Marketing roles. Outside of her science bubble, she loves to dance, sing, watch baseball and football, and go on adventures of all sorts!

Ekaterina (Kat) Pak is a fourth year PhD student in the Biological and Biomedical Sciences graduate program. For her thesis, she is studying the biology and treatment of brain tumors in Rosalind Segal’s lab at the Dana-Farber Cancer Institute. Kat graduated from MIT in 2009 as a Bachelor of Science in Biological Engineering with a minor in writing. Prior to beginning her graduate studies, Kat gained industry experience as a summer intern at the Novartis Institutes for Biomedical Research. Outside of lab, Kat enjoys jogging and photography.

Dima Ter-Ovanesyan is a second year PhD student in the Molecular and Cellular Biology Program at Harvard University. As an undergraduate, he studied biology at MIT. After college, he performed research at the Curie Institute in Paris and helped develop new cancer diagnostics at a start up biotech company in New York. For his graduate work, Dima is currently working on various aspects of RNA biology and delivery of RNA therapeutics in George Church’s lab at Harvard Medical School. Outside of research, Dima enjoys reading, cooking, running, and playing soccer.
Glossary of Important Terms:

**Therapeutics/Drugs:** Chemical or Biological substance used in the treatment, cure or prevention of disease.

**DNA:** a molecule in the nucleus of a cell that stores genetic information and instructions building proteins

**Gene:** a segment of DNA that has all of the information to make one **protein**

**RNA:** a “photocopy” of a part of DNA (a gene) used as a messenger used to make **proteins**

**Protein:** a molecule, encoded by the DNA that carries out a particular function within a cell

**Mutation:** change in DNA that causes a different protein to be made

**Central Dogma:** concept that DNA makes RNA, which makes protein in order to carry out biological functions

**Biotechnology:** engineering cells to do new things (such as making a therapeutic protein)

**Small Molecule Drugs:** bind to proteins to change their function for a therapeutic effect

**Double-Blind:** neither the patient nor the administrator knows who gets the drug vs. placebo

**High-Throughput Screening:** Testing libraries of small molecules for their ability to exert a biological or chemical change with relevance to a disease

**Rational Drug Design:** Finding new medications based on knowledge of a protein target, the entity that the drug interacts with to give a therapeutic effect.

**Biologics:** Production of biological drugs in which proteins are made to treat disease.

**Recombinant DNA:** Inserting human genes into cells that can rapidly produce large amounts of the protein

**Gene Therapy:** New type of therapeutic treatment involving the insertion of a gene (DNA) into a patient’s cells

**RNA Interference (RNAi):** A mechanism in a cell that destroys a specific RNA to be used therapeutically

**Adrenoleukodystrophy (ALD):** A lethal genetic disease that is caused by a mutation in the ABCD1 gene; leads to a loss of nerve function due to demyelination (loss of protective myelin coating surrounding brain cells)

**Blood Stem Cell:** a type of cell that can give rise to different kinds of blood cells (immune cells, red blood cells, platelets, etc.)

**Virus:** a particle that can replicate inside of cells by injecting them with RNA or DNA; used in gene therapy

**Transthyretin (TTR) Amyloidosis:** a lethal genetic disease due to a mutation in the Transthyretin (TTR) gene; misfolded TTR proteins aggregate causing tissue damage in various organs (such as heart and brain)

Upcoming SITN Events

Oct 17th, Seminar “Living Foods: The Microbiology of Food and Drink”

Oct 24th, Seminar “The Universe in a Pretty Big Box: From Cells to Galaxies Using Supercomputers

Nov 12th, Science by the Pint, Dr. James Mitchell:

“You Are What You Don’t Eat: Influencing Surgical outcome by Preoperative Diet”

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