Drug Development Milestone I

Select and characterize a disease that you plan to cure with a drug that you could design
Due date: Monday, Feb 7, 2011

As a first part of your project, you are expected to identify a disease that you want to work on. Submit two-to-three page summary where you discuss the nature of the disease, give statistics concerning its prevalence and mortality, and discuss current approaches that are used to tackle this disease. Your discussion of the nature of the disease should cover both the clinical manifestations (e.g. diphtheria, if untreated, leads to inflammation and eventual failure of the heart muscle) and important biochemical mechanisms (e.g. diphtheria toxin inactivates molecules that are critical for protein synthesis by attaching a ribosyl moiety to them)

How to go about deciding about the disease? The first step is to select a general area of drug application. Perhaps you know of a disease someone has for which the treatment is less than optimal. For example, side effects may be quite bad. Another approach is to identify an area that is broadly acknowledged as being in need of further improvement. One example is the treatment of infectious diseases (e.g., TB) that because of resistance development within the microbial strains, is in dire need of improvement. Cancer and virally transmitted diseases are some of the bigger unsolved areas. However, many less "visible" areas are interesting too. Aging, Allergic rhinitis. Sleeping sickness. Hair loss The list goes on. You could browse journals such as the Journal of Medicinal Chemistry, Biochemistry, Science, Nature, or Molecular Pharmacology to get an idea what people are working on. And of course, use the Web. Pick something that you will find fun and learn from. However, you need to remember that you need to come up with a reasonable proposal. Diseases that have not been sufficiently characterized, or diseases that are known to arise from a variety of molecular mechanisms might be too difficult for your project. As you read the literature, you will quickly develop a feeling if the disease is sufficiently well understood.

Students in the course may work alone or form a team of two when working on their project. If you feel confident in your ability to tackle such a problem alone go on with the disease of your choice. If you feel that you could benefit form a formal partnership with another student, team up. However, students who team up cannot pick their own project but shall work on one of the “feature projects” listed below. Feature projects should be tackled in the order given below; e.g. a team cannot work on cystic fibrosis unless glioma has been taken. Students who work alone are allowed to work independently on the “feature projects”. No more than one team can work on a particular feature project.

Feature projects:

- Diffuse intrinsic pontine glioma (for motivation, please see http://prayingfornina.blogspot.com/2010/08/praying-for-miracle.html)
- Cystic fibrosis
- Attention deficit hyperactivity disorder