422 2014

IGP 422 Syllabus: Summer 2014

Topic: Introduction to Translational Research

Course Master: H. William Schnaper
Co-course Master: David Johnson

Frequency and duration: Once to twice weekly for 1.5 hours Time: 4:00-5:30 pm Tuesdays and Thursdays
Location: Gray Seminar Room, Lurie 1-165

Credits: 1.0

Form: Mixture of seminar/discussion and journal club format

Purpose/Rationale: An essential focus of biomedical research is its applicability to understanding and treating human disease. This course is intended to introduce basic life sciences and clinical research graduate students to the thought processes involved in human disease research and its translation into therapy by providing an overview of disease processes, how they are treated, and how basic biological science is used to develop those treatments. At the end of this course the student should understand the medical rationale for studying basic pathomechanisms and how to utilize that rationale to design studies and grant proposals. In addition, the student will obtain background knowledge for further, disease- or organ-specific upper-level courses.

Evaluation: In the middle of the course, the students will construct a single-page outline of how they would develop a new discovery for clinical use. Before the end of the course the student will be given examples of recent discoveries based on a basic science article published within the past three years and asked to describe in 3 pages how to take that discovery to clinical application. They may request an alternative discovery subject to approval by the Course masters.

Note: The scheduling of the course sessions is intended to provide the student with some time for vacation in August, and to fit the schedules of invited speakers. So the sessions will vary on some weeks.

Sessions (all classes 4-5:30 pm. The order of the sessions is subject to change):

1. Thursday 6/26 Course introduction and Introduction to translational research  (H.W. Schnaper)
   In an overview, this session will address the concepts and principles of translational investigation. What defines translational research? What issues are important for scientists seeking to find translational implications for their work, and what issues are compelling for industry to develop those discoveries? How does a discovery go from basic science to market, and what are the impediments to this process? The role of each of the planned sessions in illustrating these concepts will be discussed.
2. Tuesday 7/1  Diabetes (W. Lowe)
The growing problem of diabetes in the developed countries. Biology of diabetes, and current translational challenges- therapeutics, transplant and regeneration.

No class 7/3 (evening before 4\textsuperscript{th} of July)

3. Tuesday 7/8  Rules and regulations in translational research and human studies  (L. Smith)
What are the basic ethical principles of translational research? How is our conduct of human studies regulated? What is the role of various government agencies in drug and device development? What is an IND and why is it necessary?

4. Thursday 7/10  Preparing a mini-proposal for translating a discovery (H.W. Schnaper)
Class exercise

5. Tuesday 7/15  Devices in Cardiovascular Medicine (M. Kibbe)
A case history: liquid stents. Developing the device through translational research, intellectual property issues, funding, working with industry, and overcoming challenges.

6. Thursday 7/17  Cancer Therapy (Shad Thaxton)
History, biomarkers, issues in drug delivery (targeting, nanodelivery systems, etc). Approaches at NU and in the lab.

\textbf{Tuesday 7/22 Midterm (single-page proposal) due.  Not a class session}

7. Thursday 7/24  Innovation in Drug Discovery and Development: Contemporary Approaches To The Design and Development of New Molecular Entity (NME) Candidates (D.M. Watterson)
This session will include an introduction to fundamental concepts and approaches used to reach rapid and definitive Go/No Go decision points in translational research focused on innovation in small molecule therapeutics discovery and discovery. Case studies from historical landmarks and on-going discovery research for novel disease modifying therapeutics will be used as didactic tools for processes applicable across disease indications.

8. Tuesday 7/29  Allergy (J. Pongracic)
Allergic disorders span the spectrum from minor irritations to debilitating and even life-threatening illnesses such as severe asthma or anaphylactic shock. This session will briefly address the cellular and molecular pathophysiology of allergic disease and then describe how basic work is being translated into active therapies useful for treatment. Potential future approaches to human allergic conditions will also be explored.

9. Thursday 7/31  Diagnostics (R. Chisholm)
Principles of diagnostics: sensitivity, specificity and other measures of relevance. How is the value of a test determined for the patient? For the doctor? For the industry developing the test? What are the principles of biomarker development and utility? New concepts of diagnostics: Genomics, proteomics, pharmacogenomics and other aspects of personalized medicine.

10. Tuesday 8/5  Translational Molecular Biology: Cystic Fibrosis (S. McColley) The gene that is mutated in cystic fibrosis, which encodes the cystic fibrosis transporter (CFTR), is the
first gene that was cloned to account for a disease. The impact of that cloning, and how it has altered our approaches to therapy for this relatively common genetic disorder, will be considered.

11. Thursday 8/7 The View from Industry (M. Schabacker, Baxter)
History, hot areas, industry-academic partnerships. How do drug companies organize to address “hot” areas? How do they decide what is important?

12. Tuesday 8/12 Drug Discovery: Ingenuity or Serendipity? (R. Silverman)
This session covers the process of how drugs are invented or discovered in general and a real-life experience of a drug (Lyrica™) being brought to market.

Thursday 8/14  Final exams due. Not a class session.