IGP/MSCI 422
Syllabus: Summer 2015

**Topic:** Introduction to Translational Research

**Course Master:** H. William Schnaper, schnaper@northwestern.edu

**Co-course Master:** David Johnson, davej@northwestern.edu

**Teaching Assistant:** Daniela Janevska, DanielaJanevska2012@u.northwestern.edu

**Day/Time:** Tuesday and Thursday; 4:00 - 5:30 pm.

**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, how basic biological science is used to develop those treatments, and the role of various stakeholders in the translational research pipeline. 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.

**Student Evaluation:**

Readings/Class discussion (10%): Prior to each class session, students will be asked to read the articles. These articles will be discussed in class and that discussion, along with interactions during the seminars, will be evaluated for this portion of the grade.

Midterm (30%): Students will be provided with a recent primary article and asked to write a single-page outline of how they would develop a new discovery for clinical use. Further instructions will be provided in class.

Final (60%): Student will be provided with examples of recent discoveries based on a basic science article (different from midterm) 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. Further instruction will be provided in class.

**Required Readings:** Readings for each session will be posted on Canvas.

**Academic Integrity:**

Every Northwestern faculty member and student belongs to a community of scholars where academic integrity is a fundamental commitment. The Program in Public Health abides by the standards of academic conduct, procedures, and sanctions as set forth by The Graduate School at Northwestern University. Students and faculty are responsible for knowledge of the information provided by The Graduate School on their Web page at http://www.tgs.northwestern.edu/about/policies/academic-integrity.html
Academic misconduct includes, but is not limited to
1. Receiving or giving unauthorized aid on examinations or homework
2. Plagiarism
3. Fabrication
4. Falsification or manipulation of academic records
5. Aiding or abetting any of the above

The PPH follows The Graduate School’s procedure for evaluating alleged academic misconduct, as outlined on the TGS website.

****Note: The scheduling of the course sessions is intended to provide the students with some time for vacation in August, and to fit the schedules of invited speakers.

**Sessions:** Each session will consist of a 10- to 15-minute student lead discussion and a invited guest speaker lecture (all classes 4-5:30 pm. The order of the sessions is subject to change):

1. **Tuesday 6/23** Course introduction (Schnaper/Johnson)
   Introduction to Clinical and Translational Research (D. Lloyd-Jones)
   After a brief introduction to the course and its requirements, 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? What impediments prevent the translation of discovery to markets? What is the role of the translational research infrastructure at Northwestern?

2. **Thursday 6/25** Developing a Specific Immune Tolerance Therapy for Multiple Sclerosis: Mouse Meets Man (S. D. Miller) NOTE: For this class only, we will meet in the Kellerman Classroom on the 2nd floor of McGaw Building.
   Dr. Miller has worked for over 30 years on the basic science of autoimmune disorders (in particular, multiple sclerosis). It is only recently that his findings have been applied to human diseases, and he will discuss his experiences in going from bench to bedside to startup company.

3. **Tuesday 6/30** Rules and regulations in translational research and human studies (L. Smith)
   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?

No class Thursday 7/2 (evening before 4th of July holiday on 7/3)

4. **Tuesday 7/7** 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 the challenges of having a startup company.

5. **Thursday 7/9** Cancer Therapy and the Northwestern “Incubator” (C. S. Thaxton, N. Maull)
   CS Thaxton will cover the history of drug development for cancer, companion cancer diagnostics, current drug development strategies and why they fail, and new approaches to cancer drug development that take advantage of nanotechnology. N. Maull will discuss the role of NU in facilitating discovery development, and working with faculty startups
Monday 7/13  Midterm assignment due (single-page outline of how the student would develop a discovery for practical use)

6. Tuesday 7/14  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.

7. Thursday 7/16  The View from Industry  (Norton, Abbvie)
History, hot areas, industry-academic partnerships. How do drug companies organize to address “hot” areas? How do they decide what to invest in?

8. Tuesday 7/21  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.

9. Thursday 7/23  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.

10. Tuesday 7/28  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.

11. Thursday 7/30  Ethics in Clinical and Translational Research  (M. Moran)
Major errors in judgment both abroad and in the United States, and perhaps the evolution of our sensibilities, have led to altered understanding of the rights and responsibilities of investigators, subjects, patients and the public with regard to clinical research. This session will review that evolution and consider the roles each of the stakeholders has in the translational research process.

12. Tuesday 8/4  The Future of Biomedical Research and Treatment: Personalized Medicine  (R. Chisholm)
New concepts of diagnostics: Genomics, proteomics, pharmacogenomics and other aspects of personalized medicine. How the revolution in molecular medicine is changing the way we treat patients.

Thursday 8/11  Final paper due.  Not a class session.