Abbreviated Draft Syllabus

MEDI 501: Principles of Preclinical Translational Science: A Case Study from Cancer Drug Discovery and Development

Fall 2022 Semester (October 26–December 13, 2022; 7 weeks)

Course learning objectives:

- Understand the definitions and goals of translational research and translational science, and how they differ.
- Identify a range of scientific and operational principles of translational science that can be applied to enhance preclinical translational research projects.
- Learn about the research process necessary to enable a scientific discovery to lead to an effective compound that can be used in humans.
- Learn about the varied roles of different disciplines and agencies -- including industry, government agencies, and academia -- in advancing translational research, and how to facilitate effective inter-agency and team-based collaboration.

Week 1: Overview of the Course, Translational Science, and Initiation of this Project

Orientation Lecture: Introduction to MEDI 501 Principles of Preclinical Translational Science: A Case Study from Cancer Drug Discovery and Development (Jessica Faupel-Badger)

See email for invitation to complete pre-course survey

Lecture 1a: Translational Science: Maximizing the Success of Translational Research (Joni Rutter)

Lecture 1b: Translational Science Principles (Jessica Faupel-Badger)

Lecture 1c: Challenges in Development of Selective Anti-Metastasis Therapies in Today's Cancer Treatment Landscape (Udo Rudloff)

Lecture 1d: Pursuing Novelty to Accelerate Innovation in Translational Research (Juan Marugan)

Assignments: Introduction assignment, mini-quiz, assigned reading and submit questions for office hours.

Week 2: Optimizing Efficiency and Effectiveness in Translational Research: Infrastructure, Teams and Partnerships, and Scientific Approaches

Lecture 2a: Collaborative Discovery at NCATS Early Translation Branch (ETB) Part 1 (Matt Hall)

Lecture 2b: Collaborative Discovery at NCATS Early Translation Branch (ETB) Part 2 (Matt Hall)

Lecture 2c: Identification of a phenotypic marker for selective anti-metastasis drug development (Sui Huang)

Lecture 2d: *In vitro* Assays for Drug Discovery and Development: Towards Better Clinical Predictability (Marc Ferrer)

Lecture 2e: Using Phenotypic-Based Drug Discovery Approaches to Discover Anti-Metastatic Drugs (Marc Ferrer)

Assignments: Mini-quiz, 2-minute paper, assigned reading and submit questions for office hours.

Week 3: Cross-Disciplinary Teamwork and Cross-Agency Partnerships to Advance Translation

Lecture 3a: Medicinal Chemistry in the Preclinical Translational Research Team (Sam Patnaik)

Lecture 3b: Medicinal Chemistry Approaches in the Metarrestin Project (Sam Patnaik)

Lecture 3c: Partnering for Success, Part 1: Principles and Management of Intellectual Property (Krishna "Balki" Balakrishnan)

Lecture 3d: Partnering for Success, Part 2: Approaches for Effective Collaboration (Krishna "Balki" Balakrishnan)

Assignments: Mini-quiz, assigned reading and submit questions for office hours**.**

LIVE office hours TBD Lecturers will answer questions submitted in advance, and reserve time for a few live questions. More information forthcoming, including information on how to join live or view the recording.

Week 4: Evidence-Based Practices to Enhance Team-based Collaboration, Creativity and Innovation in Translational Research

Lecture 4a: Strategies for Effective Team Interactions: Evidence Based Practices from the Science of Team Science Field (Amanda Vogel)

Lecture 4b: Planning for Success in Team Science (Amanda Vogel)

Lecture 4c: Team Science in the Metarrestin Project: Group Interview (Amanda Vogel)

Lecture 4d: The Division of Preclinical Innovation (DPI) Systems Approach to Creating and Sustaining a Team Science Environment (Ann Knebel)

Lecture 4e: Creativity in Science Teams: What is it, and how do you achieve it? (Roni Reiter-Palmon)

Lecture 4f: Fostering Innovation in Science Teams: Team and Organization Conditions (Roni Reiter-Palmon)

Assignments: Mini-quiz, 2-minute paper, assigned reading, and submit questions for speakers/office hours.

Week 5: Advancing Along the Translational Spectrum: Predictive Models in Drug Development; Pharmacology and Toxicology Testing in the Preclinical Research Project

Lecture 5a: Uses, Strengths, and Limitations of Preclinical Cancer Models, Including Animal Models, for Predicting Future Response in Humans (Udo Rudloff)
Lecture 5b: Pancreatic Cancer Overview and Aligning Animal Models with Clinical Needs for the Metarrestin Project (Udo Rudloff)

Lecture 5c: Transitioning from Discovery Research to IND Enabling Studies (Phil Sanderson)

Lecture 5d: Application of Pharmacokinetics in Preclinical Translational Research (Xin Xu)

Lecture 5e: The Role of Toxicology Data in Filing for an Investigational New Drug (IND) (Pramod Terse)

Assignments: Mini-quiz, assigned reading and submit questions for office hours.

Week 6: Target Identification

Lecture 6a: Principles for Target Identification in Phenotypic Drug Discovery Efforts (Juan Marugan)

Lecture 6b: Principles for Target Identification in the Metarrestin Project (Juan Marugan)
Assigned reading and mini-quiz

Assignments: Mini-quiz, 2-minute paper, assigned reading and submit questions for office hours.

LIVE office hours, TBD. Lecturers will answer questions submitted in advance, and reserve time for a few live questions. More information forthcoming, including information on how to join live or view the recording.

Week 7: Regulated Clinical Trials and Course Wrap-Up

Lecture 7a: Clinical Trials Goals, Design and Implementation, Part 1 (Elizabeth Ness)

Lecture 7b: Clinical Trials Goals, Design and Implementation, Part 2 (Elizabeth Ness)

Lecture 7c: Update on Design and Status of NCI 20-C-0023: First-in-Human Phase I Trial to Investigate the Safety, Tolerability, Pharmacokinetics, Biological and Clinical Activity of Metarrestin (ML-246) in Subjects with Metastatic Solid Tumors **(**Udo Rudloff)

Lecture 7d: Course Conclusion and Additional Resources (Jessica Faupel-Badger)

Assignments: Mini-quiz and assigned reading.