TRx Boot Camp

DATE/TIME: Every Monday from Jan 23 – Feb 27th, 4:00pm -6:00pm (no class on Feb 20)

LOCATION: 622 W 168th St., PH building, 10th floor, room 405 A/B

COURSE OUTLINE: The TRx Boot Camp is a 5- session course designed to give academic investigators an overview of certain aspects of the drug development and commercialization pathway. The lecture series will provide an overview of the drug development process with a special focus on rare disease indications and how strategies differ with varying therapeutic modalities (small molecule, large molecule, gene therapy, cell therapy, etc.).

Upon completion of the boot camp, attendees will be able to position their therapeutic discoveries for future commercial value.

Boot camp attendance is mandatory for investigator teams that have submitted pre-proposals and are interested in submitting a full proposal for the TRx Resource Pilot Award but open to all Columbia faculty, staff, and students.

COURSE SCHEDULE:

	Topic	Presenter
Session I **January 23**	**Market Research & Competitive Analysis**	**Lorraine Marchand, MBA** ***Centricity Health***
Session II **January 30**	**Intellectual Property, Licensing, and Sponsored Research**	**Teresa Chen, PhD, JD** ***Columbia Office of General Counsel*** **Ron Katz, PhD, MBA** ***Columbia Technology Ventures***
Session III **February 6**	**Developing a Target Product Profile**	**Randall Kaye, MD** ***SSI Strategy***
Session IV **February 13**	**Experimental Design with Translational Perspective**	**Anton Simeonov, PhD** ***NCATS,NIH***
Session V **February 27**	**Working with the FDA**	**Dilcia Granville, PhD** ***US FDA***

CONFIDENTIALITY: We ask all participants to read and adhere to the following Confidentiality Statement:
"As a participant in this course, I understand that my fellow participants may wish to pursue patent protection for their course projects and that confidentiality is often essential for doing so. In order, to promote a free exchange of information among participants, I shall treat all communications on course

projects that I receive from participants during the time period of the course as confidential. I shall not disclose these communications to any third party, except for that which is already known or rightfully obtained by me prior to its disclosure to me by the participants or other advisors."

Session Description and Goals

1. Identifying Unmet Need: *Market Research and Competitive Analysis*

- Goals: What is market research and why is it important in early research? How can you access market data? What is an addressable market? How to identify the competitive landscape.
- Rare disease: what is the business model for rare diseases? Does a therapeutic need to target a rare disease as well as a large market disease to be valuable?

2. Intellectual Property: *Inventions, disclosures, licensing and sponsored research*

- Goals: What is a disclosure and why be careful of disclosures? When does a research, project become intellectual property? Is there a difference in IP strategy for different therapy types? When should you contact the tech transfer office? What are the paths to commercialization (licensing, option, SRA)?
- Rare disease: What is unique when commercializing therapeutics with rare disease indications? What are value-add tools (vouchers, patent strategies and market exclusivity)

3. Developing a Target Product Profile: *Changing mindset to focus on future product*

- Goals: What is a TPP/TMP? How does Industry use TPP/TMP? What is the value of a TPP for early stage research? When should you pivot? What criteria are important to consider?
- The TPP is project specific not disease state specific

4. Experimental Design: *Design of preclinical studies with commercial perspective*

- Goals: What type of experiments do you need to do for pre-clinical data? What work can be done through outsourcing to a CRO, NIH/NCATS or within the academic institution? How do you work with third parties? What resources does Columbia have available to support investigators? How to design experiments that demonstrate feasibility? "fastest route to failure"
- Rare Disease: what pre-clinical experiments are not required for rare disease therapies?

5. Regulatory Information: *Description of Pre-IND meetings, regulatory pathways*

- Goals: How can academic investigators begin to work with the FDA? At what point should an investigator reach out to the FDA? What are the different pathways for regulatory approval? What is the general timeline?
- Rare Disease: What is the path for therapeutics that have a rare disease indication? Description of FDA programs for Orphan/rare diseases.