COURSE DESCRIPTION

Regulatory science concerns “the development and use of tools, standards and approaches to more efficiently develop products and to more effectively evaluate product safety, efficacy and quality”. With the increasing role of regulatory agencies as knowledge stewards and broker’s (data sets), comes the expansion of the regulatory mission, from solely protection, to the protection and promotion of the health of the public.

Innovation to address the 21st century’s evolving regulatory landscape, in the context of new technologies, new understanding of diseases and a sharpened lens on safety -- is an exciting new area of science, one that requires a new generation of trained regulatory experts and professionals in academic, government, industry and public health sectors.

The aim of the Regulatory Science, Drug Development and Public Health Online Course is to provide students with information needed to understand the most important health practice and product regulation issues in the US and abroad from the perspective of current regulatory standards, their standards for evidence and the role of innovation in regulatory science.

The course is of interest to participants in regulatory agencies, public health, law, medicine, business or policy interested in biotech and the pharmaceutical industry, working in the settings of industry or regulatory agencies. As the biomedical industry continues to rapidly grow, the demand for professionals in the regulatory sciences does so as well, nationally, bi-nationally and globally.

INSTRUCTOR INFORMATION

Veronica Miller is a leading expert in the process of advancing regulatory science and facilitating a development path through collaborative engagement of stakeholders from both sides of the Atlantic. She has extensive experience in addressing regulatory issues in several key disease areas, working closely with experts from the US Food and Drug Administration (FDA) and the European Medicine Agency (EMA) in these projects.
Dr. Miller is the Executive Director of the Forum for Collaborative Research, a public and private partnership addressing cutting edge science and policy issues through a process of stakeholder engagement and deliberation. Dr. Miller is also Professor (Adjunct) at the UC Berkeley School of Public Health. She developed and teaches a course on FDA and Drug Development based on case studies from the Forum's rich history in facilitating drug development. She mentors interns and fellows pursuing regulatory, biotech, and translational medicine careers. Dr. Miller has published over 100 peer-reviewed publications on treatment and regulatory strategies.

**COURSE OBJECTIVES/GENERAL COMPETENCIES**

By the end of the course, participants will be expected to be able to:

- Demonstrate knowledge of the basic concepts of a regulatory path, regulatory science, innovation and ethical principles, and their application in areas of public health need from a global, regional and national perspective.
- Identify, assess, think critically about the basic tenets of regulation in public health settings, including infectious diseases and regulation of controlled substances.
- Explain and critically evaluate the clinical research process and assess the underlying mandate for evidence, for benefit and for risk, at each stage of the process.
- Describe and apply regulatory science concepts and be able to think critically about the role for innovative regulatory science for specific health products, including small molecules, biologics, vaccines, medical devices and diagnostics, cellular tissue and genetic engineering.

**COURSE SCHEDULE**

| Introduction to Regulatory Science and Regulation |
|---|---|
| **Topic 1** | (a) Introduction to the Course |
| | (b) Introduction to Regulatory Science |
| | Audio Lecture (Miller) |
| | Interview: Janet Woodcock FDA |
| **Topic 2** | Regulatory Approaches in Different Countries/Regions |
| | Interview series: Hans-Georg Eichler EMA; Mac Lumpkin Bill & Melinda Gates Foundation; Lahouari Belgharbi COFEPRIS; Mouna Akacha – comments on ICH |

**Readings**


### International Public Health and Regulation

| Topic 1 | Ethics in Clinical Research  
Audio lecture (Miller) |
|---------|----------------------------|
| Topic 2 | International Collaboration and Treaties  
Audio lecture (Drs. Maria Cecilia Acuna & Jean-Mark Gabastou - PAHO) |
| Topic 3 | Infectious Diseases and Global Health Emergencies  
Interviews: Jeffrey Murray FDA; Michelle Berrey Chimerix; Cliff Lane NIH |

#### Readings


### Drug Development Pathway

| Topic 1 | Steps along the Continuum  
Audio Lecture (Miller) |
|---------|-------------------------|
| Topic 2 | From Proof-of-Concept to Efficacy and Effectiveness (Miller)  
Case study 1: HCV Cure  
Interview: Filip Josephson, EMA  
Case study 2: Prevention of HIV Acquisition  
Interview: Charu Mulick DAVP CDER FDA  
Case study 3: Non-Alcoholic Fatty Liver Disease  
Interview: Laurent Fischer Allergan; Elmer Schabel EMA |
| Topic 3 | Standards of Evidence and Approaches to Design and Analysis  
Audio Lecture (Miller)  
Interview: Mouna Akacha “Estimands Concept”  
Hans-Georg Eichler: Clinical Trial Design interview |

#### Readings

**Required:** (Miller and Grant 2014) (Hutchison, Kwong et al. 2014) (Dunn and Glidden 2016) (Friedman, Sanyal et al. 2016) (Sanyal, Friedman et al. 2015)

**Optional:** (Miller and Woodcock 2017) (Sanyal, Neuschwander-Tetri et al. 2016)
| Topic 1 | Surrogate Markers vs Hard Clinical Endpoints  
Case study 1: HIV  
Case study 2: HBV  
Audio lecture (Miller)  
Interview: Jeff Murray FDA; John Sninsky #2; |
|-----------------|---------------------------------------------------------------|
| Topic 2 | Expedited Approval Mechanisms and Early Access in US and EU  
Case Study 1: CMV, ADV  
Case Study 2: HCV  
Audio Lecture (Miller)  
Interview: Michelle Berrey (#1)  
Interview: Elmer Schabel, EMA  
Class discussion Trogarzo case study |
| Topic 3 | Biomarkers, Context-of-Use and Qualification Pathway  
Audio lecture (Miller)  
Interview: Chris Leptak FDA |

### Readings


**Optional:** (Donaldson, Harrington et al. 2015) (Eichler, Oye et al. 2012) (Kesselheim and Darrow 2015) (Lauer and D’Agostino 2013) (Sarpatwari, Darrow et al. 2015)

### Assessing Benefit-Risk; Long-term Safety

| Topic 1 | Benefit-Risk in Context - not a static formula  
Special Considerations for Pediatric Populations, Rare Diseases  
Audio Lecture (Miller)  
Interviews: Michelle Berrey; John Crowley (Amicus); Ilan Irony (FDA); Seema Shah (University of Washington) |
|-----------------|---------------------------------------------------------------|
| Topic 2 | Pharmacovigilance in the US, Europe and across the World  
Audio Lecture (Miller)  
Audio Lecture (Tobias Peschel Gilead)  
Interview: Nyasha Bakare Janssen |

### Readings

### Regulatory and Technological Innovation

#### Topic 1
Regulatory Science Innovation:
Patient Centered and Patient Focused Clinical Research; Platform Trials
Audio Lecture (Miller)
Case study: PSC
Interviews (Ricky Safer PSC Partners; Martine Walmsley PSC Support; Rob Myers Gilead)

#### Topic 2
Frontiers in Technological Innovation:
Gene and Cell Therapy
Audio Lecture (Miller)
Interviews: Steven Gray (University Texas); Susan Nichols (Falcon Therapeutics); Kathy High (Sparks); Anne Pariser (NCATS)

### Readings

**Required:** To be confirmed

**Optional:** To be confirmed

### Regulation of Biologics and Vaccines

#### Topic 1
Regulation of Biologics
Audio Lecture (Miller)
Interview: Rachel Witten FDA-CBER;

#### Topic 2
Regulation of Vaccines
Audio Lecture (Miller)
Interview: Robert Janssen (Dynavax); Julie Gerberding (Merck); Philip Krause (FDA CBER)

### Readings

**Required:** TBD

**Optional:** (TBD)

### Medical Devices

#### Topic 1
Regulation of Medical Devices in the International Context: Challenges and
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READINGS


Fauci, A. S. (2016). "No more excuses. We have the tools to end the HIV/AIDS pandemic." Washington Post.


FDA (2015). "Targeted Drug Development: Why are many diseases lagging behind?".

FDA (2017). "Discussion Paper on Laboratory Developed Tests (LTDs)."


Plotkin, B. J. and M. C. Hardiman (2013). Infectious Diseases Surveillance and International Health Regulation, John Wiley & Sons Ltd.


