There has been a growing interest in using real-world data (RWD) and evidence (RWE) in drug development and regulatory science since the passage of the 21st Century Cures Act in December 2016. The US Food and Drug Administration released the Framework for FDA’s Real-World Evidence Program in December 2018 and subsequently issued in May 2019 a draft guidance for industry on Submitting Documents Using Real-World Data and Real-World Evidence to FDA for Drugs and Biologics. The recently issued RWE guidance by China National Medical Product Administration (NMPA) encourage sponsors to use RWD and RWE in clinical trial design and analysis and regulatory submission. This short course will discuss the evolving field of RWD and RWE with the focus on how RWD and RWE can be more efficiently used in the design and analysis of clinical trials, statistical and causal inference methodologies for generating RWE from RWD for regulatory submissions, and challenges and opportunities in using RWD and RWE in the regulatory setting.

Table of Contents
1. Introduction
2. Real-world data and clinical trial data
3. Finding “fit-for-purpose” real-world data
4. “Substantial evidence” to support regulatory decision
5. Estimands in real-world data and evidence
6. Control for confounding variables in the design and analysis of clinical trials
7. External data to serve a control group for single-arm trials
8. External data to augment concurrent control group in randomized trials
9. Design and analysis of Pragmatic clinical trials
10. Phenotyping and enrichment of the RWD for effective leveraging of RWD for the RWE generation
11. Review of current state of electronic phenotyping of RWD
12. Statistical learning and its incorporation for RWD enrichment
13. Other considerations in the design and analysis of clinical trials using external data
14. Causal inference frameworks for real-world evidence generation
15. Regulatory considerations in using real-world data and evidence for decision-making
16. Challenges in practical use of the RWD
17. Best practice in using real-world data and evidence
18. Challenges and opportunities