Practical and Challenging Issues in Regulatory Science

**Abstract:** During drug and biologic product development, some practical and challenging issues are inevitably encountered. These issues have an impact on the process of regulatory review and approval. These issues include, but are not limited to, (i) the use of 90% confidence interval approach for generics/biosimilars versus the use of 95% confidence interval approach for new drugs, (ii) the selection of study endpoints, (iii) the selection of non-inferiority (similarity) margin, (iv) sample size requirement, and (v) design and analysis for rare diseases clinical trials. Most recently, United States Food and Drug Administration (FDA) has kicked off several critical clinical initiatives to not only improve but also to shorten drug and biologic product development. These clinical initiatives include big data analytics, real world data/evidence, complex innovative design (including adaptive design and n-of-1 trial design), model-informed drug development, biomarker development for precision medicine, master protocols in cancer research. This presentation intends to cover practical and challenging issues that are commonly seen in regulatory review and approval process and introduce FDA clinical initiatives regarding drug research and development.

**Keywords:** Endpoint Selection, Margin Selection, Real World Data/Evidence; Complex Innovative Design (CID), Master Protocols, Model-informed Drug Development