Use of Regulatory Flexibility to Accelerate Drug Development

Chairs:
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Disclosures

Kemi Olugemo: ISCTM Scientific Committee; Former employee/stockholder at Korro Bio, Ultragenyx, and Ionis

Sian Ratcliffe: ISCTM Executive Committee; Employee of Premier Research; Former employee of Biogen; Former employee and shareholder of Pfizer

Why this session is needed

- ~ **1 in 7 to 1 in 10** drug candidates that start human testing make it to market.
- The cost of drug development continues to skyrocket – creating unsustainability
- There is an urgent need to create more efficiency in the drug development process, and to accelerate the speed of drug development
- Innovative trial methods with real examples of trials approved/discussed with FDA and EMA
- Methods applicable to CNS and other therapeutic areas

Session Topics

Path to prevention (P2P) therapeutics **platform trial** in early neuronal synuclein disease
Use of **external controls** for the primary analysis of a randomized phase 3 SMA study
Prognostic adjustment and other **digital twin methods** for improving trial efficiency
Accelerated approvals based on a **surrogate endpoint** in Alzheimer's disease
Precision medicine at scale: Operationalizing **individualized trials**
Panel/Regulatory Discussion

Speakers

Roland Brown, PhD
Tianle Chen, PhD
Christopher Coffey, PhD
Emily Freilich, MD
Richard Foster, MSc
Sarah Glass, PhD
Kemi Olugemo, MD, FAAN
Siân Ratcliffe, PhD