Blueprint for Innovation: Charting the Future of FDA Policies

Joga Gobburu,
University of Maryland, Baltimore

Purposes of Guidances

Encourage Innovation

Clarify Regulatory Expectations

Procedural

Promote Consistency

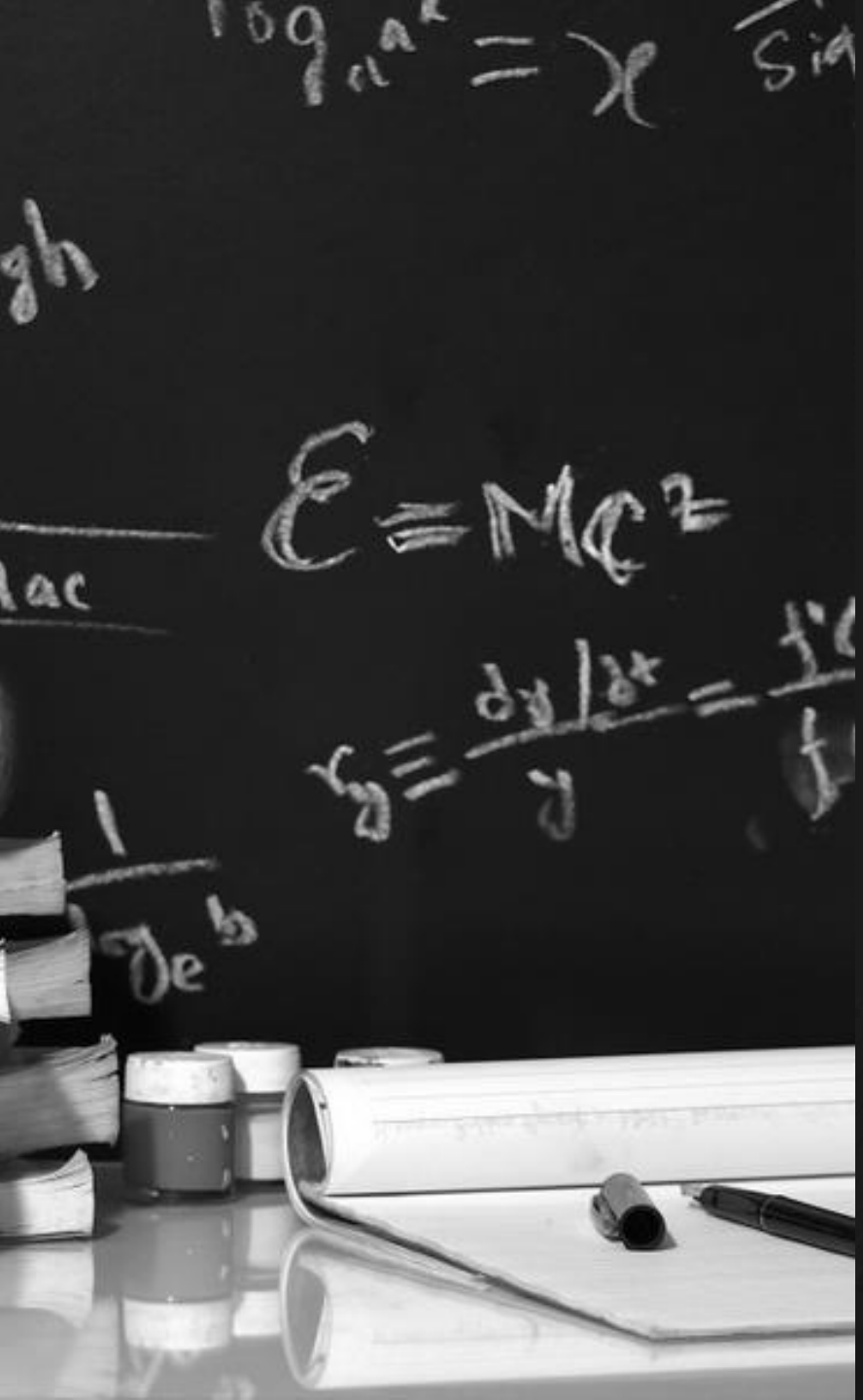
Purposes of Guidances

Encourage Innovation

Clarify Regulatory Expectations

Procedural

Promote Consistency



Topic#1

Demonstrating Substantial Evidence of Effectiveness using Model-based Endpoints

Conventional group comparison may not be applicable.

E.g. rare disease, special populations

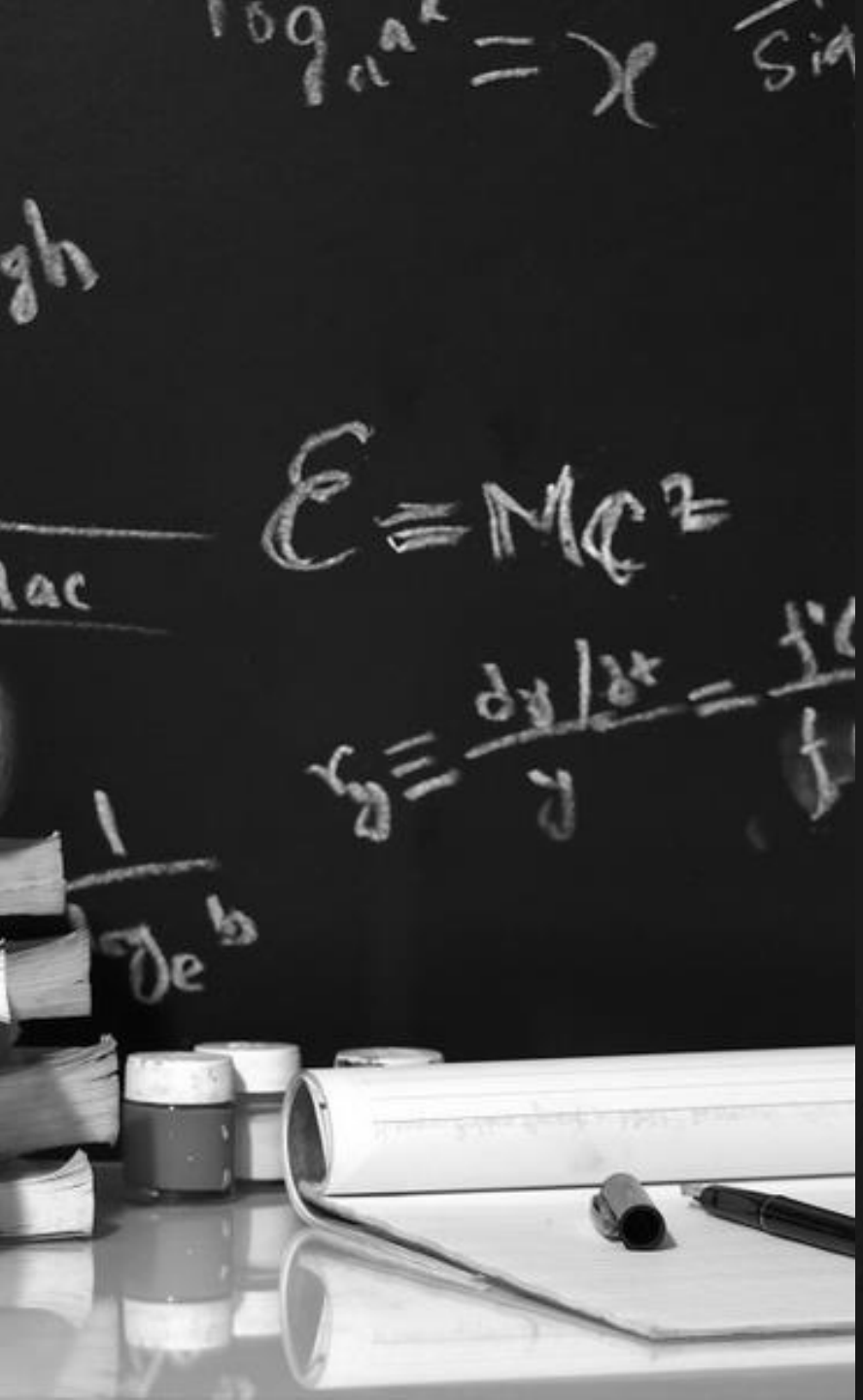
Encourages more efficient use of data.

E.g. dose selection, individualization of treatment

Leverage more informative trial designs.

E.g. Encourage dose-ranging trials

Demonstrating Substantial Evidence of Effectiveness using Model-based Endpoints



Topic#2

Demonstrating Substantial Evidence of Effectiveness using Pharmacodynamic Endpoints

Biomarker unrelated to MOA.

E.g. rare disease, special populations

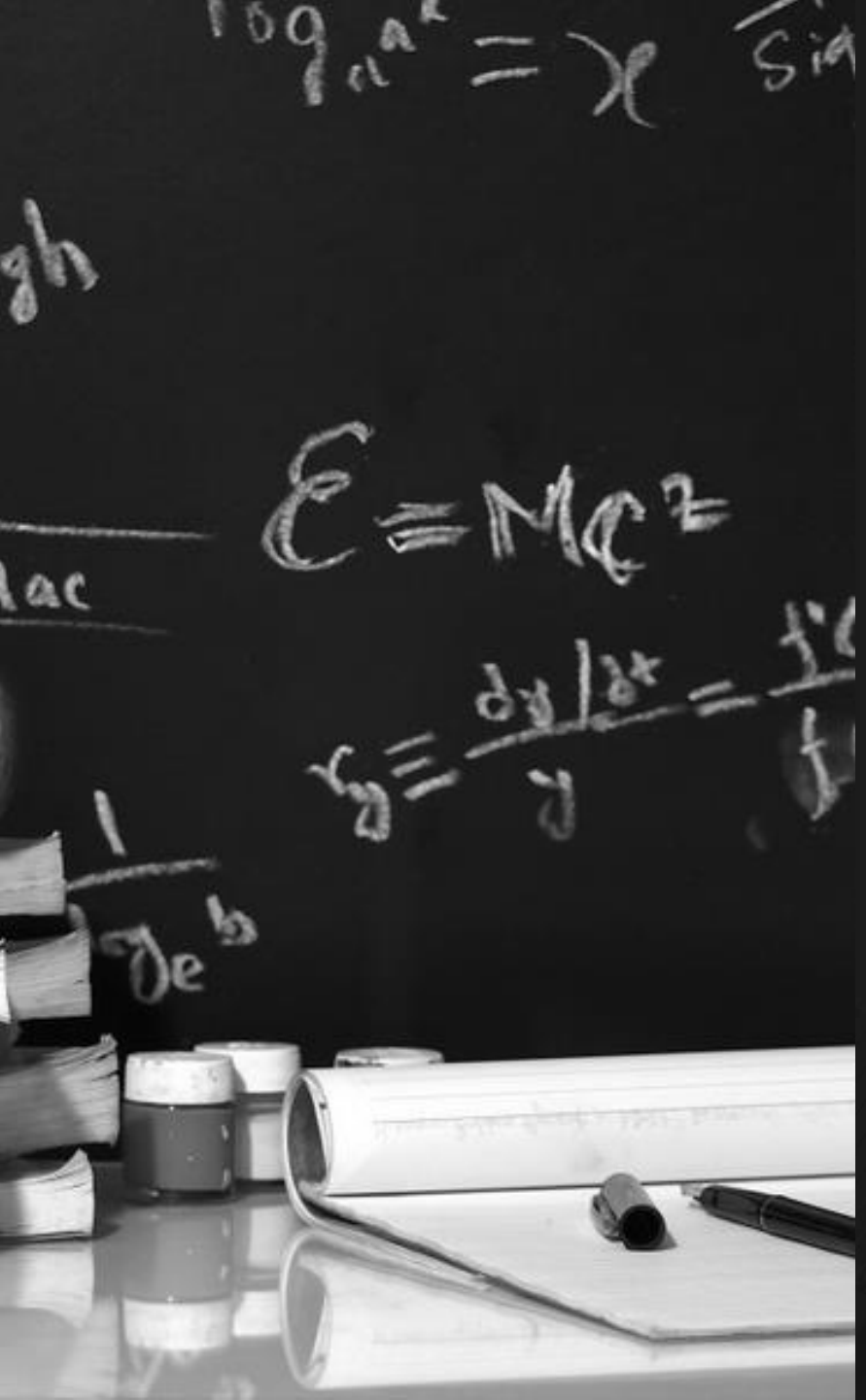
Potential use in aiding dose selection or adjustments.

E.g. dose selection, individualization of treatment

Encourage innovation in methodology

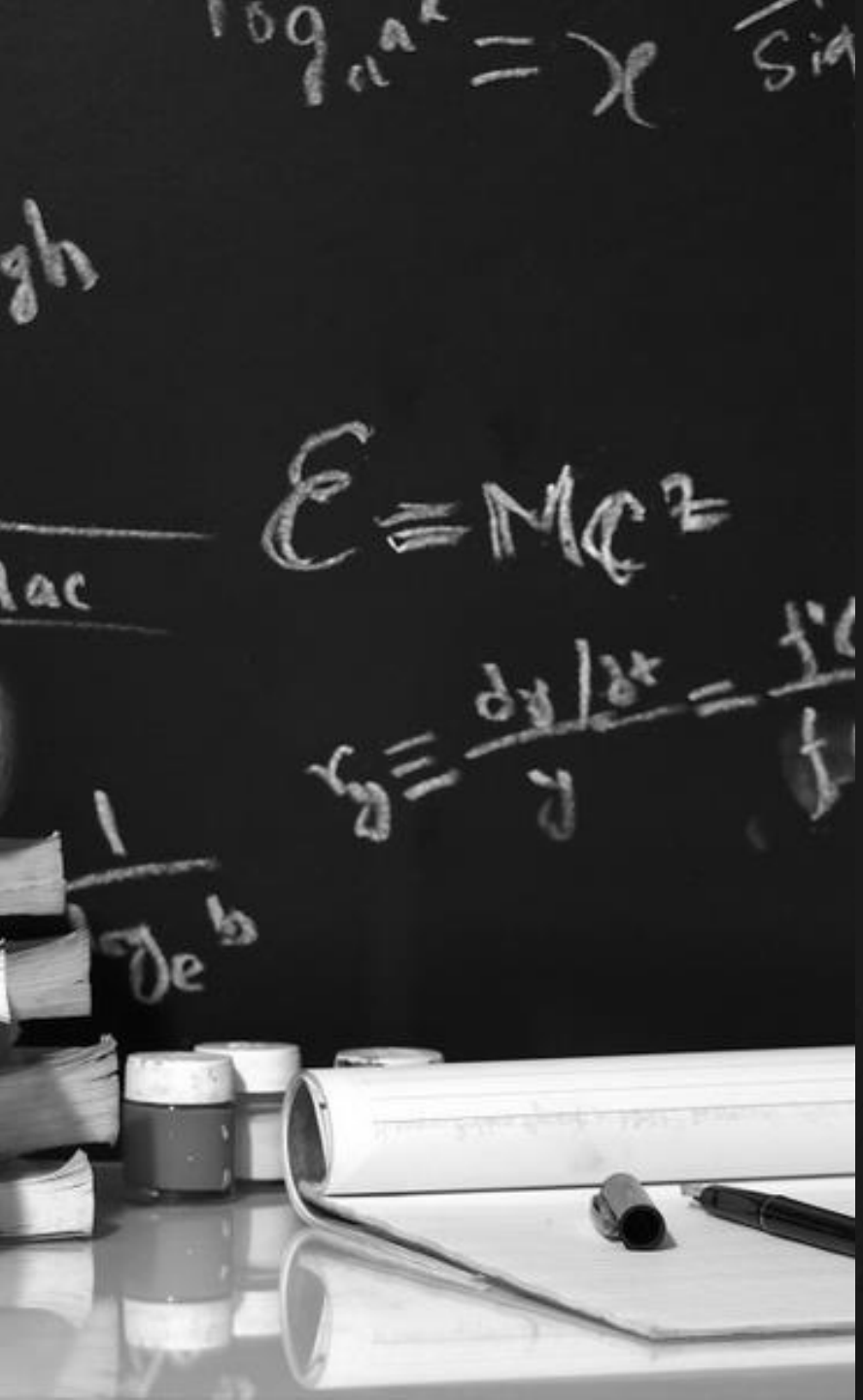
E.g. criteria for identifying predictive PD markers

Demonstrating Substantial Evidence of Effectiveness using Pharmacodynamic Endpoints



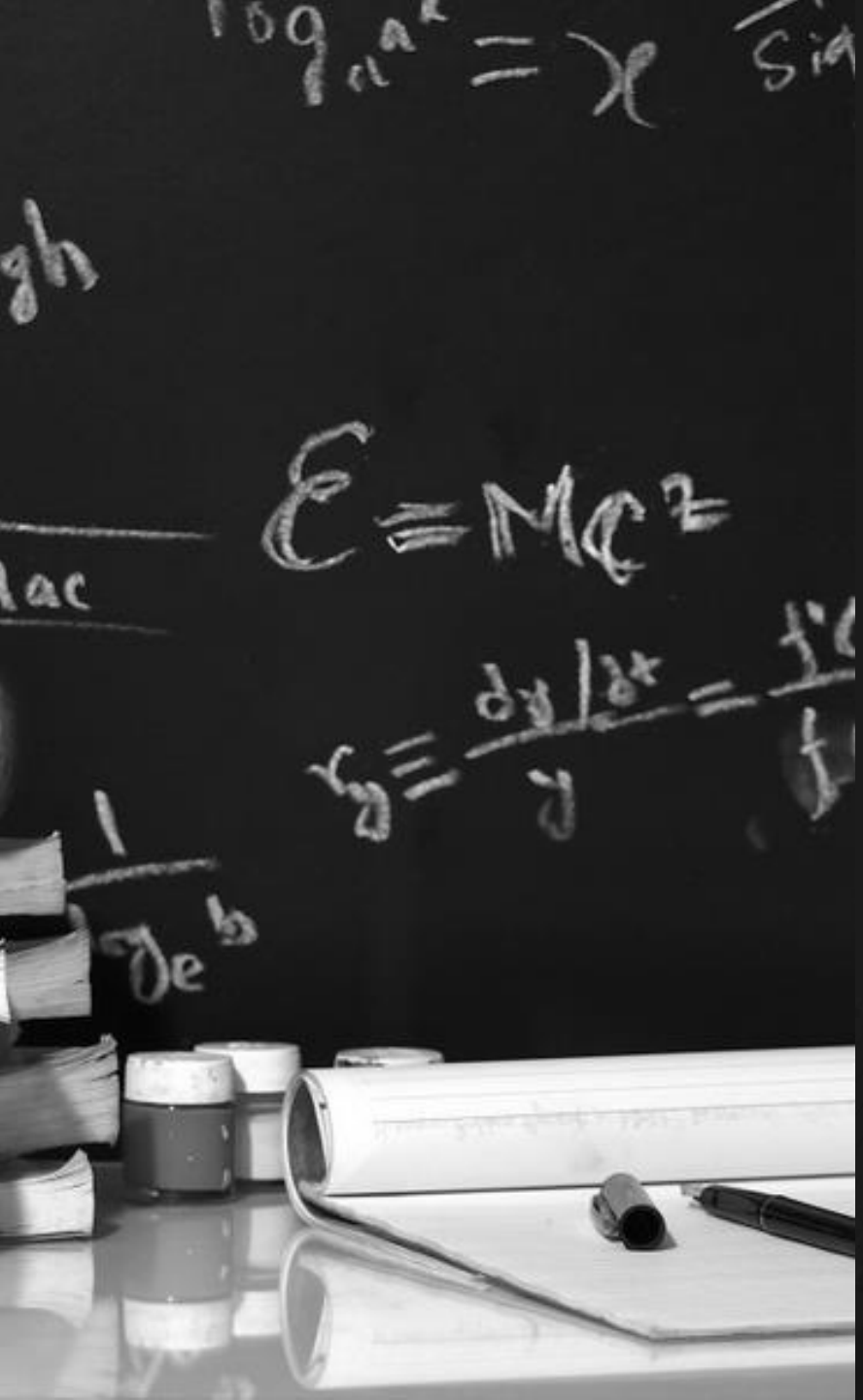
Topic#3

Pharmacokinetic-Based Criteria for Supporting Alternative Dosing Regimens of Programmed Cell Death Receptor-1 (PD-1) or Programmed Cell Death-Ligand 1 (PD-L1) Blocking Antibodies for Treatment of Patients with Cancer



Topic#3

Pharmacokinetic-Based Criteria for Supporting Alternative Dosing Regimens and Dosage Forms



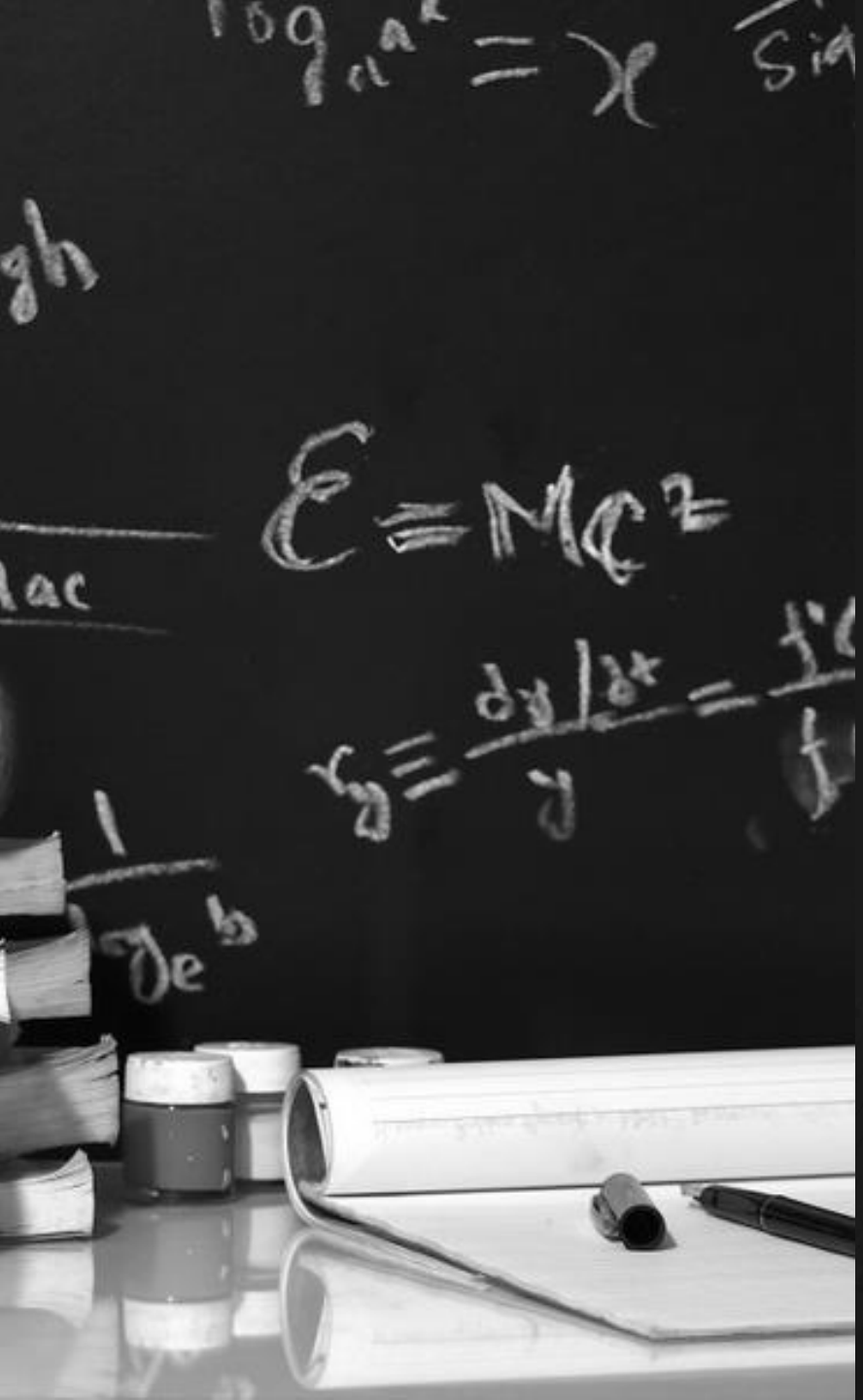
Topic#4

Applications of Machine Learning to Supporting Drug Development and Regulatory Decisions

General considerations in ML Model Development and Validation.
E.g. methodology, validation, and testing of ML models

Integration with Regulatory Decision Making.
E.g. assessment of drug efficacy and safety, and support personalized medicine

Data Standards and Interoperability.
E.g. standardization of data formats and protocols to enhance the interoperability of ML models



Topic#5 (initiative)

Project Sync

Labels do not reflect the latest scientific & clinical knowledge

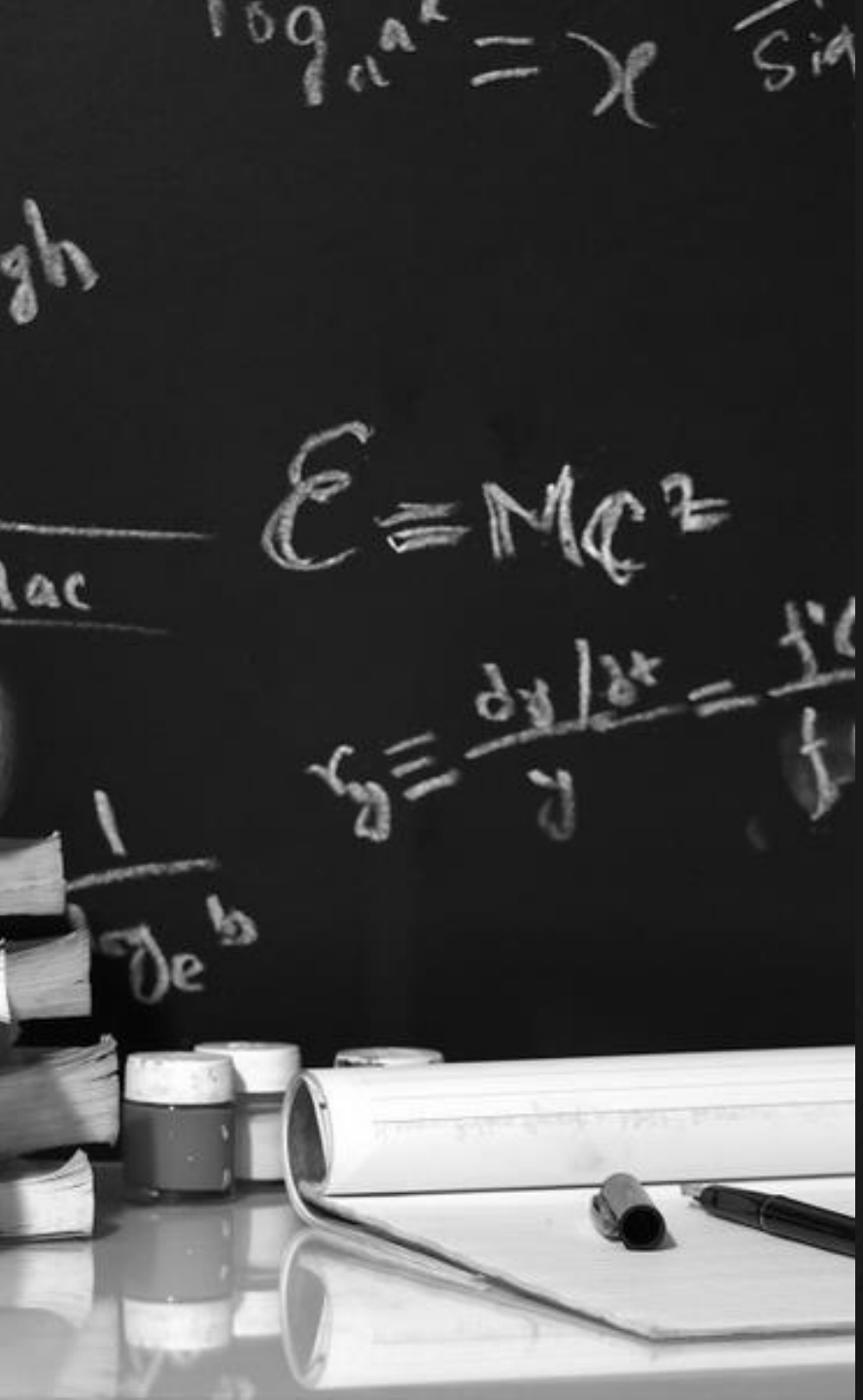
E.g. several published studies are not informing drug usage

Access to Real World Data allows gaining insights into individualized Pharmacotherapy

Opportunity to engage stakeholders

E.g. research and training to build stronger workforce

Project Sync



Topic#6

Evidentiary standards for supporting algorithms for individualizing pharmacotherapy

RCTs to support individualized pharmacotherapy are impractical

E.g. data from conventional RCTs and others can support individualization algorithms

Algorithms can form the scientific basis for devices

Opportunity to engage stakeholders

E.g. research and training to build stronger workforce

Project Sync

Blueprint for Innovation: Charting the Future of FDA Policies

Joga Gobburu,
University of Maryland, Baltimore