Track 6 | Translational Sciences and Precision Medicine

Preclinical and early-phase clinical research provides initial dosing and safety data for new drugs. This track focuses on the latest strategies used in early-stage compound selection, updates on safety considerations for both drugs and biologics, how PK/PD affects dosing strategies, and methods to improve data quality and integrity for proper downstream decision-making.

DIA recommends this track and associated sessions to professionals involved in pharmacology and toxicology, nonclinical safety testing, clinical research, clinical operations, safety and pharmacovigilance, project management, patient centricity, and statistics; formulation science, pharmacokinetics/pharmacodynamics, epidemiology, toxicology, and regulatory affairs.

Included Topic Areas

Personalized medicine, clinical trial data disclosure, collaborations, bioethics, compliance, stem cells, regenerative therapies, cell and gene therapies, gene editing, organoids/micro-physiological systems, ICH (S), study endpoints, integration of the ‘patient’s voice’ early in preclinical development to define/refine the patient population and clinical endpoints, preclinical studies, and challenges in rare and common diseases. Topics related to bioethical issues are also welcome and may be considered for a special track in the meeting.

Priority Topics

1. Innovations in Early Development of Vaccines: Translation from Pre-Clinical to Clinical
   a. Pre-clinical and early clinical program of vaccine products—differences for cancer vaccines versus infectious disease vaccines
   b. Translatability of animal data to human data
      i. Meaningfulness and predictivity of the selected pre-clinical animal model for the human in vivo system
      ii. Optimizing pre-clinical approaches to ensure data quality and informativeness
   c. Leveraging pre-clinical data (in vitro, in vivo) to predict dose in human
   d. Quantitative relationship between dose/dosing schedule and immune response
   e. Pathogen resistance to vaccines and treatment and emergence of new strains (AI opportunities to evaluate and assess impact, strategies for overcoming pathogen resistance); regulatory landscape and considerations for vaccine development around the world—have we lowered the bar for approval?
   f. Leveraging lessons learned during COVID-19 vaccine development to inform pre-clinical decisions that lead to efficiency in clinical conduct
   g. Lymphadenopathy (LAP) in vaccine development—development challenges and safety considerations

2. Diversity, Equity, and Inclusion in Early Drug Development
   a. Clinical and scientific importance of diversity in omics studies for discovery science and early clinical development

3. What’s New in Gene Therapy
   a. Leveraging AI to predict complications in vector integration
   b. Understanding durability of effect gene therapy
   c. Considering the patient journey
   d. Pre-clinical models for reliable prediction of efficacy and toxicity
   e. Existing regulatory frameworks and challenges for pre-clinical development and early phase clinical trials

4. What’s New in Gene Editing
   a. Recent advances in the field
   b. Off-target editing—estimating, predicting, and interpreting impact on treatment
   c. Existing regulatory frameworks and challenges for pre-clinical development and early phase clinical trials
   d. Pre-clinical models for reliable prediction of efficacy and toxicity

5. Innovative New Models and Methods for Medical Product Development
   a. Accelerating pediatric therapeutic development
   b. Model informed drug development
      i. Leveraging in silico technology to predict toxicity and safety risks
      ii. Value of QSP models to facilitate key decisions in drug development

6. Early Development Decisions and Mitigating Challenges in Rare Disease Drug Development
   a. Regulatory considerations and decision-making when the mechanism of action is not understood
   b. Prolong new drug life cycle with 505(b)(2) path—optimizing early decision-making to avoid product development failures
   c. Challenges on translational aspects of pre-clinical findings and how to expedite these.

7. Precision Medicines in Early-Phase Clinical Development
   a. Strategies for precision dosing
   b. The use of novel technologies and overcoming scientific and regulatory challenges
   c. The development and use of biomarkers and companion diagnostics

8. The Microbiome Factor in Drug Discovery and Development