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 vaccines 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
   - b. Strategies, best practices, and case examples to include more diversity in early drug development to advance discovery and pre-clinical work
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
   - c. 3D organ models for novel pre-clinical testing
   - d. Artificial intelligence and digital technologies in support of preclinical or early clinical 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**