



2023 Program Themes



Discovery and Basic Research

Theme 1: Advancing Therapeutic Discovery and Development: Mechanistic Considerations for Drug Delivery

Keywords: SLC transporters, ABC transporters, nucleic acid vectors, nanoparticles, antibody-based delivery, receptor-mediated transcytosis, cell-based delivery.

One of the many challenges in drug discovery and development is the ability to deliver a therapeutic to its site of action at effective concentrations. Over the past several years, new modalities have been designed to ensure successful cell/tissue drug delivery. These paradigms include, but are not limited to, transporters, nucleic acid delivery vehicles, nanoparticles, antibody-based drug conjugates, and cell-based delivery systems. This theme will provide state-of-the-art information as to how drug delivery should be considered early in the drug development process and stimulate high-level discussion on next-generation drug delivery modalities. Discussion will also focus on biological mechanisms that enable state-of-the-art drug delivery platforms to be deployed for improved treatment of human disease.

Theme 2: Addressing Health Disparities in Basic Science Research

Keywords: aging, sex differences, racial/ethnic differences, pharmacogenomics, drug metabolism, drug transporters, toxicokinetics, gene and cell-based therapies, rare diseases, precision medicine.

Recent advances in pharmacokinetics and pharmacodynamics have revealed considerable differences in drug ADME properties, drug response, and toxicokinetics amongst human populations. Such differences can potentially lead to serious issues such as adverse drug events, unexpected drug-drug interactions, and poor treatment effectiveness. This theme will explore strategies for incorporation of genetic differences based on sex, race, and ethnicity into early-stage drug discovery campaigns. Additionally, evaluation of diseases and/or comorbidities in basic science studies will be discussed. The theme's objective is to use this critical information to provide guidance for academic, industrial, and regulatory scientists in addressing health disparities in their research. This information that will lead to the development of safer and more effective pharmacological treatments for human disease.

Theme 3: State-of-the-Art Tools for Basic Research and Early-Stage Drug Discovery

Keywords: Imaging modalities, organoids, stem cell systems, organ-on-a-chip, metabolomics, proteomics, in vivo disease models.

In conjunction with the rapid pace of early stage discovery science is the advent of new tools for use in basic research projects. These tools have included new imaging modalities for the study of drug disposition; stem cell systems to generate more robust in vitro models; organoid/organ-on-a-chip models that incorporate cell-cell communication to incorporate more complex models into research approaches; and new animal models that better incorporate the complexities of human disease. Additionally, generation of “-omics” data sets have provided considerable information that can guide drug discovery. Of particular importance is understanding how these research tools can be leveraged for more efficient drug discovery and to accelerate investigation of new pathways or molecular targets for therapeutics. This theme will focus on the methodologies themselves and how data derived from these laboratory tools can best be used to facilitate discovery and development of new and improved medicines.



Preclinical and Translational Sciences

Theme 1: Predictive Biomarker Strategies to De-Risk Drug Development

Keywords: Predictive biomarker, DDI, DILI, PK/PD.

Translational researchers are advancing more predictive and efficient strategies with an aim to predict, reduce, and nullify potential risk in drug development from early drug discovery through late-stage clinical trials. Alteration of drug-metabolizing enzymes or drug transporters can have a significant effect on drug disposition and exposure, leading to drug-drug interactions (DDIs) or drug-induced organ toxicity, which can lead to termination of candidate drug development. Predictive biomarkers can be useful in early drug development to assess drug-induced organ toxicity, DDIs, and to understand PK variations in subjects with genetic polymorphism or organ impairment. PD biomarkers can be useful to inform drug response and dose selection. Some PD biomarkers can ultimately be used for approval. For example, the recent approval of Aducanumab relies heavily on biomarker of PET imaging for amyloid plaque change. This theme covers the application of advances in technology and techniques that discuss

the discovery and qualification of predictive biomarkers for PD, DDIs, patient selection, and organ toxicity.

Theme 2: Advancing Innovation in In Silico Translational Approaches

Keywords: Translational sciences; in silico, PK/PD, modeling and simulation, PBPK, MIDD, biologics, immunogenicity, new modalities, AI/machine learning.

The objectives of this theme are to discuss advancement and application of in silico quantitative approaches in the preclinical phase to post-approval to support development of new targets/modality, reduce the use of animals, improve clinical study design, and optimize therapeutic individualization. For example, in silico modeling and simulations can be very useful in supporting recent FDA Project Optimus, emphasizing characterization of dose-response for oncology.

Submissions are encouraged to cover research and development that enable advancing translational science approaches such as: in silico modeling and simulation; use of machine learning to inform clinical trial design; physiological based PK modeling applications (e.g., bridging formulations, biowaivers); first-in-human dose prediction for novel modalities that do not have good preclinical models; and the translational aspects of preclinical immunogenicity data to humans. Case studies translating application of such tools to the clinic are encouraged.

Theme 3: Novel Predictive Preclinical Models for Efficacy and Toxicology

Keywords: In vitro assay, in vivo model, MPS (microphysiology system), efficacy, toxicology, translation, prediction.

Successful clinical drug development relies on effective preclinical testing. Large-scale or mechanism-based in vitro testing and the ability to combine proteomic, metabolomic and other novel technologies can speed up the development of more effective and safer drugs. Preclinical testing is constantly evolving especially with advancements in biotechnology resulting in development of novel modalities and increased understanding of disease and drug metabolism mechanisms. Further, FDA Modernization Act 2.0 may lead to changes in nonclinical testing and strategies, which could inspire new approaches for in vitro assays and models to predict clinical efficacy/safety. Some examples of new preclinical models are patient-derived xenograft models, 3-D organoids in cancer therapy development, and organ-on-a-chip for potency and toxicity evaluation. This theme will share cutting-edge preclinical testing systems for better translation of preclinical drug metabolism, efficacy or toxicity data to the clinic.



Bioanalytics

Theme 1: Setting up for Success -- Considerations for Study Design in the Context of Bioanalysis

Keywords: Microsampling, patient-centric sampling, tissues, flow cytometry, biomarkers, surrogate biomarkers, immunogenicity, multidomain therapeutics, bispecific, CGT (cell and gene therapy), DDI (drug-drug interactions).

This theme incorporates a range of considerations related to bioanalytical sampling and strategy paired to downstream bioanalysis: patient-centric sampling and the impact on bioanalysis; considerations for tissues, blood samples, and non-standard matrices; the integration of biomarkers into a bioanalytical program, and considerations for their use and qualification; selection of approaches for immunogenicity testing for multidomain or complex molecules, including selection and generation of positive control reagents; selection of molecular approaches for cell and gene therapy trials; and the impact of interferents and concomitant medications.

Theme 2: Approaches for Emerging Modalities and Complex Studies

Keywords: Troubleshooting, CGT (cell and gene therapy), immunogenicity, critical reagents, stability, multidomain therapeutics, bispecifics, LC-MS, PCR, molecular, oligonucleotides, RNA therapeutics.

This theme will examine new technologies and considerations for complex therapeutics, and approaches for troubleshooting challenging issues in the bioanalytical lab and in the conduct of complex trials. Topics include advances in molecular techniques; hybrid approaches for small molecule/complex therapeutic analysis; new approaches for data analysis; assay development and maintenance in support of cell and gene therapy programs; assay development and maintenance for multidomain therapeutics; case studies of analysis for non-standard therapeutics; critical reagent management and reagent bridging; approaches for stability for non-standard modalities; and troubleshooting-focused case studies.

Theme 3: The Bioanalytical Lab of the Future

Keywords: Automation, high-throughput, surrogate matrices, animal models, supply chains, regulatory, quality, outsourcing

This theme explores the industry, regulatory, and technological trends that are shaping the bioanalytical lab and the field of bioanalysis. Topics will include: advances and incorporation of automation; high-throughput approaches; outsourcing and capacity in the field of bioanalysis; integration of surrogate matrices; use and implications of new animal or non-animal models; supply chain constraints; speed and timelines related to drug development; companion diagnostics; impacts of new regulatory guidance; and new statistical approaches; and quality considerations.



Manufacturing and Analytical Characterization

Theme 1: Advanced Manufacturing and Emerging Technologies in Pharmaceuticals and Biopharmaceuticals

Keywords: Continuous manufacturing, adaptive supply chains, flexible modular manufacturing, accelerating development, crystal and particle engineering, form stability and phase transformations, automation, ICH Q12 / Q13, post-marketing change management, sustainability, operational excellence.

Continuous manufacturing for small and large molecule DS/DP including control strategy considerations, design space; demand-adaptive, flexible supply chains; portable, modular, and point-of-care manufacturing; rapid response manufacturing and analytical strategies for accelerating development; integrated manufacturing platforms connecting drug substance and drug product; novel crystal and particle engineering strategies; co-processed API; form stability and process-induced phase transformations; Industry 4.0/Pharma 4.0; digital manufacturing; automation in manufacturing and analysis; emerging regulatory expectations, ICH Q12 / Q13; post-marketing change management and comparability (including scale up/scale out, shelf-life extension program); sustainability, green processes, biocatalysis. Additionally, operational excellence topics including facilities of future, process optimization, and validation.

Theme 2: Advanced Modelling and Predictive Approaches in Drug Development, Manufacturing and Analysis

Keywords: Predictive design, process modelling, system models, digital twins, PAT, material characterization, machine learning and analytics, multivariate modeling, real time monitoring, lights out manufacturing.

Mechanistic and data-driven process modelling for unit operations; modelling of devices; product performance modelling and in silico predictions; system modelling; digital twins; application of machine learning in development and manufacturing; materials science and material sparing predictive tools; molecular modelling; crystal structure predictions to predict manufacturability; AI-based image analytics and microstructure characterization; MSPC models in manufacturing; PAT and real-time release; PAT for biopharmaceuticals, real-time monitoring; Lights-out manufacturing and release by exception; automated QA/QC.

Theme 3: Novel Modalities and Cross-Modality Considerations in Manufacturing and Analysis

Keywords: Manufacturing and analysis of new modalities, ADCs, adjuvants, vaccines, LAIs, bioenhancement, novel excipients, enabling analytical technologies, analytical QbD approaches in novel modalities, analytical characterization of ADCs, analytical development of novel excipients and adjuvants, impurity characterization, nitrosamines, particulates, regulatory and quality considerations for novel modalities and novel excipients

Manufacturing, analysis and novel excipients for new modalities and emerging delivery systems (mRNA delivery, lipid based systems, oligonucleotides, ADCs, adjuvanted vaccines, microneedles, implants, etc.); manufacturing and analysis of long-acting injectables including IVIVC strategies; bioenhancement, amorphous DP manufacturing and analysis; dissolution and release of novel dosage forms; novel enabling analytical technologies; modality agnostic topics around analytical QbD, analytical life-cycle management, and risk assessment approaches; control strategies for process related impurities, particulates, contaminant controls; nitrosamines; extractables and leachables; analytical challenges at the interface of modalities such as characterization of bioconjugates of small and large molecules; control strategies for cross-modality products and novel platforms; regulatory and quality considerations for novel modalities and novel excipients.



Formulation and Delivery

Theme 1: Accelerating the Drug Development Process through Formulation and Delivery Strategies

Keywords: Critical quality attributes, AI, modelling, site and disease specific drug delivery, acceleration.

Formulation and delivery strategies that can accelerate the drug development process may largely improve efficiency and shorten the time to market for patients in need. The theme covers a variety of strategies and current advancements on accelerating formulation development, such as high throughput screening; critical quality attribute identification; advanced evaluation tools for early-stage development; developability assessments; risk-balanced development strategies; AI and modelling tools to enable formulation development; in vitro and in vivo correlation; site- and disease-specific drug delivery; platform processes and technologies; multifunctional micro- and nano-delivery systems; modified release; long-acting implants; and related topics, for chemical, biomolecular, and new modality drugs.

Theme 2: Enabling Excipients and Approaches for Advancing Formulation and Delivery

Keywords: Novel excipients, formulation technologies, material properties, physicochemical and biological properties.

The increasing complexity of new molecular entities drives the need for enabling excipients. Excipients impact a wide range of performance characteristics. This theme explores enabling excipients such as new small-molecule, lipid, polymeric, and peptidic excipients to serve as enteric coatings; permeability enhancers; mucoadhesives; enzyme inhibitors; transport enhancers; cell penetrators; tight-junction modulators; complex formulation stabilizers; and related topics. The theme also covers the excipients for implantable delivery devices and drug-device combinations and formulation technologies that are used based on the types of excipients -- i.e., microencapsulation, spray drying, nanoencapsulation technologies, excipient material properties, and drug-excipient physical and chemical interactions.

Theme 3: Formulation and Delivery for Patient Centric Product Development

Keywords: Patient centricity, high concentration formulation, drug-device combination, target product profile (TPP).

Innovative patient-centric development has been increasingly explored to enhance patient convenience, comfort, and compliance. This theme includes formulation and development strategies that can address technical challenges and lead to the ultimate clinical success of patient-centric products, such as drug-device combination products (e.g. autoinjectors or patch pumps) enabling patient self-administration; delivery technologies allowing subcutaneous delivery of large volumes; and non-intravenous routes of administration (e.g. intranasal, pulmonary, or intradermal). The theme also includes strategies for efficient introduction of novel formulation and delivery technologies to deliver patient-centric products at market launch, such as enhanced communication between functional areas; early implementation of the quality target product profile process; and mutual understanding of the different technical challenges encountered between discovery, clinical, CMC organizations, and patients, among others.



Career Development

Track: Navigating the Business of Science

Theme 1: Navigate or Develop Business Acumen to Advance a Scientific Career

In today's competitive world, scientists need to gain a better understanding of business to make better decisions and build successful careers. A well-developed technical set of goals coupled with business knowledge prepares scientists to become leaders in tomorrow's environment. Examples of topics relevant to this theme include: negotiating a contract, understanding stock options, protecting intellectual property, elements of job offers, managing a division or company, securing resources to fund scientific and/or business goals, managing investor relationships in light of company goals, inter-company negotiation during partnerships and collaborations (in-licensing, mergers and acquisitions, co-developed assets), getting started as an entrepreneur, and securing venture capital funds for a start-up.

Theme 2: Achieve Work-Life Harmony and Build a Sense of Unity for a Diverse Group

Globalization has brought professionals from diverse backgrounds together to achieve common objectives. A successful team benefits from differences in education, training, experiences, cultures, and the contribution of ideas by its members. Examples of topics relevant to this theme include: how to foster meaningful diversity to enrich a workforce, understanding unconscious bias and its effect on decision-making, ways to support mental health in a competitive environment, effective listening practices for leaders, how to use inclusive language, building a diverse network and support system in the workplace, strategies for expanding one's skills, saying no without guilt, and the role of sponsorship in improving organizational diversity.