2024 Program Themes

Discovery and Basic Research

Theme 1: Leveraging AI in the Processes of Drug Discovery, Lead Validation, Preclinical Development, and Regulatory Submissions

Keywords: Artificial Intelligence (AI), Bioinformatics, Drug Discovery, Drug Repurposing, Machine Learning (ML), Metabolomics, Predictive Network Analysis, Proteomics, Regulatory Guidelines, Target Identification.

Artificial Intelligence (AI) is playing a critical role in the metamorphosis of drug discovery and development -- expediting processes, refining decision-making, and optimizing resource allocation. The integration of AI is transforming diverse phases of drug discovery, lead validation, and drug development by introducing innovative solutions for augmenting efficiency, precision, and swiftness during the preclinical stage. Furthermore, AI and Machine Learning (ML) have enabled the repurposing of currently marketed drugs toward novel disease indications. AI-based approaches also include integration of "-omics" data sets into the drug discovery process, thereby enabling predictions of novel therapeutic targets and indices of drug effectiveness. The overarching objective of this theme is to impart firsthand insights into approaches related to target identification and validation, AI-based compound screening, on biological activities, and predictive model development. Specific emphasis will be given to predicting adverse effects. The theme will focus on how predictive modeling can be harnessed to recognize new therapeutic indications for difficult-to-treat and rare diseases. Attention will be directed toward strategies to expedite preclinical discovery steps in alignment with the evolving requirements from regulatory submissions.

Theme 2: Challenges and Opportunities Associated with Targeted Therapeutics

Keywords: Antibody-Drug Conjugates, Cancer, Drug Delivery, Infectious Diseases, Intracellular Signaling, Molecular Pharmacology, Neurological Diseases, Precision Medicine, Targeted Therapeutics, Transporters, Receptor-Mediated Transcytosis.

Targeted therapy has demonstrated improved efficacy and safety in a diverse array of medical conditions, particularly cancers, neurological diseases, and other ailments characterized by molecular or

genetic irregularities. The application of targeted therapeutics has now been extended to include functionalized antibodies and protein substitution, as well as small molecule and protein-based approaches for treating and mitigating human genetic and acquired diseases. Additionally, gene editing and RNA-based therapeutics, such as SiRNAs, microRNAs, CRISPR, and antisense oligonucleotides (ASOs) directly target the mechanisms responsible for producing disease-associated proteins. It is noteworthy that targeted modulation of immune responses has been successfully integrated into cancer vaccines and the management of various pathogenic infections. The overarching aim of this theme is to furnish the audience with state-of-the-art advancements in drug targeting and precision medicine for the development of novel approaches to treat human diseases.

Theme 3: Innovative Methodologies for Advancing Therapeutics from Bench to Bedside

Keywords: DNA Microarray, Drug Metabolism, Drug Transport, Imaging Modalities, Large Molecule Drugs, Organ-on-a-Chip, Pharmacokinetics, Single-Cell Technologies, Small Molecule Drugs.

The progression of therapeutics from the laboratory bench to the patient's bedside represents a multifaceted and dynamic undertaking, necessitating the deployment of innovative methodologies to rigorously ensure efficacy, safety, and the seamless translation of scientific breakthroughs into tangible clinical applications. Within this context, emphasis is placed on advanced tools and technologies that significantly contribute to the development of both small- and large-molecule drugs. Cutting-edge imaging modalities, such as positron emission tomography (PET) and magnetic resonance imaging (MRI), along with theranostic approaches as diagnostic tools to identify specific biomarkers or disease characteristics, as well as the targeted delivery of therapeutic agents, are now routinely utilized in early-stage development of innovative therapeutic drugs. Additionally, DNA microarrays play a crucial role in understanding the genetic basis of diseases and drug responses. Organ-on-a-chip platforms replicate the microenvironment and physiological conditions of human organs, offering insights into pharmacokinetic properties of novel therapeutics. Furthermore, single-cell techniques provide valuable information on the therapeutic efficacy and metabolite profiling of new drugs, especially when cell heterogeneity is a concern.

Preclinical, Clinical, and Translational Sciences

Theme 1: Translating the Translational Sciences - Different Understanding, Common Aim

Keywords: Translation, Preclinical Pharmacology, Clinical Development, Biomarkers, Patient Outcome.

Translational science is a multi-disciplinary field that focuses on applying knowledge from basic scientific research to practical advancements in healthcare. Translational sciences play a crucial role in expediting the transformation of scientific insights into tangible benefits for patients, such as new drugs, therapies, and diagnostic tools. With that, this theme explores different types of translations that form the backbone of successful drug discovery, development, and implementation. These include molecular and genetic translation, bridging the gap between preclinical studies to clinical research (Phase 1, Phase 2, Phase 3), and moving from clinical trials to implementation in real world and global settings, ultimately leading to improvement in patient outcomes.

This theme brings together scientists involved across the spectrum of translational research to compare and discuss novel approaches, practical applications including case studies, and areas where such tools have been used successfully in practice.

Theme 2: Making Data Work for Us – Applying and Combining Empirical (AI/ML) and Mechanistic Modeling in the Preclinical, Clinical, and Post-Approval Realms

Keywords: Modeling and Simulation, PBPK/QSP, Mechanistic Models, Empirical Models, AI/ML, Digital Biomarkers.

This theme explores the gaps in making AI/ML successful in delivering breakthrough medicines and/or approaches/discoveries in the drug development field and debates the need to apply combined approaches to empirical and mechanistic modeling.

This theme covers includes: virtual data / synthetic data / synthetic control (at preclinical and clinical levels); replacement of placebo/vehicle control in preclinical trials with computational models, replacement of control group in clinical trials with computational models; data quality and datasets size and heterogeneity and its influence on the model quality, large datasets, Real World data and its use; empirical and mechanistic (PBPK/QSP) models combined to solve problems – i.e. virtual BE studies; use of virtual data to dynamically adapt trial designs in real-time based on accumulated information.

Theme 3: Advancing Vaccine Development: Utilizing the Power of Model-Based Approaches

Keywords: Model informed drug development, vaccines, immunogenicity prediction, dose optimization, QSP, real world data

Model-informed drug development is a key tool that is broadly used to support drug discovery and development as well as regulatory review. This theme centers on the application of model-informed approaches in vaccine development and will highlight the strategic integration of clinical pharmacology tools, including dose/exposure-response analyses, mechanistic PK/PD modeling, model-based meta-analysis, and other quantitative approaches to address challenges and inform decision-making across all stages of vaccine development. Applications will include (but are not limited to) optimization of dosing regimen and formulation components, informing dosing for special populations, prediction of immunogenicity, and leveraging real world data for refinement (e.g. variants, long term safety).

Our aim is to bring together scientists from industry, academia and regulatory agencies in a collaborative dialog that encourages the adoption of quantitative tools leading to the accelerated development of more effective and safer vaccines.

Bioanalytics

Theme 1: Bioanalytical Lab Innovations

Keywords: Automation, Artificial Intelligence (AI), Machine Learning (ML), High Throughput, Assay Design, Predictive Outcomes, Devices, Platforms, Supply Chain, GLP, GCP, Clinical, Drug Development, Troubleshooting, Validation, Bioanalytical, Therapeutic, Robotics, Cut-Point Analysis.

This theme explores new technologies -- including Artificial Intelligence (AI) and Machine Learning (ML) – and their applications to bioanalysis. It also explores platforms, robotics, and automation solutions for advancing therapeutics in the bioanalytical lab. Topics include integration of robotics, microfluidics, and automation in high throughput platforms; assay design; automated sample processing; adaptation of 96 well assay format to 384 well format --case studies of development, validation, and analysis for non-standard therapeutics; solutions for GLP and GCP lab documentation using AI technologies; and innovative lab solutions for troubleshooting complex therapeutics.

Theme 2: Current Challenges in Bioanalytical Labs

Keywords: PK, PD, ADA, APA, NAb, Assays, Immunogenicity, PCR, Molecular, RT-PCR, dd-PCR, ELISPOT, Potency Assays, Domain Specificity, CAR-T, Cross-Validation, Biodistribution, Cell and Gene Therapy, ADCs, AOCs, Oligonucleotides, RNA Therapeutics, Biologics, Multidomain Therapeutics, Critical Reagents, Genomics, Stability, Matrices, Surrogate, LCMS, LBA, Troubleshooting, Microsampling, Con-Conforming Modalities, Cut-Point Analysis.

This theme explores current challenges for the bioanalytical lab's assay development, validation, analysis, and assay lifecycle management. Topics include the selection of platforms for genomics-based molecular assays; challenges for cell and gene therapy -- unique issues to the development of the ADCs, AOCs, and multi-domain therapeutics; hybrid approaches for the small molecule/complex therapeutic analysis; stability challenges and critical reagent management for complex therapeutics; matrix selection; nontraditional samples in matrices such as whole blood, tissues, PMBC, cerebrospinal fluid etc.; patient-centric sampling and bioanalytical challenges and approaches for personalized medicine; and troubleshooting-focused case studies.

Theme 3: Regulatory Implications for Bioanalysis

Keywords: PCR, Molecular, RT-PCR, dd-PCR, ELISPOT, Domain Specificity, CAR-T, Cross-Validation, LCMS, LBA, Study Design and Implementation, Cell and Gene Therapy, Immunogenicity, Gaps, Regulatory, Biomarkers, Clinical, Assays, Rare Diseases, Animal Models, Alternate Animal Models, Microsampling, Sample Logistics (China HGRA).

This theme explores the implications of the gaps in the current regulatory guidelines and incorporation of industry recommendations. Topics include biomarkers, hybrid LC/MS, cell and gene therapies and

nucleic acid analysis; incorporation of automation and Artificial Intelligence (AI); selection and testing of unique or rare matrices; cut point for rare diseases; outsourcing; study design and implementation through application of current guidance and industry white papers; harmonization across regulatory authorities or where gaps in guidance exist; impacts and implementation of new regulatory guidance; and quality considerations when guidance is undefined.

Manufacturing and Analytical Characterization

Theme 1: Innovation in Pharmaceuticals/Biopharmaceuticals Manufacturing and Characterization

Keywords: Continuous manufacturing, single use technologies, modality agnostic flex facilities, green chemistry, sustainability, collaborative ecosystems, smart manufacturing, bioprocessing innovation, pharmaceutical manufacturing 4.0, EU regulatory reform, cross-industry collaborations, disruptive innovation, industry-academia collaboration, federal funding for innovation, digital replicas, predictive modeling, ICH Q12 and Q 14, capillary zone electrophoresis, isoelectric focusing, ion exchange, size exclusion (SEC), reversed phase (RP) or hydrophilic interaction liquid chromatography (HILIC), coupled with ultraviolet (UV), fluorescence, or mass spectrometric (MS), LC MS/MS, SEC-MS, Drug release and dissolution.

The pharmaceutical and biopharmaceutical industries are experiencing a wave of innovations that are revolutionizing their traditional manufacturing and characterization. These innovations include continuous manufacturing, single-use technologies, flexible facilities, modality-agnostic manufacturing systems, and modular manufacturing systems including use of robotics. Additionally, there is a growing emphasis on green chemistry and sustainability in pharmaceutical and excipient industry.

Characterization techniques have evolved commensurate to the complex nature of novel formulations and manufacturing processes. These advanced techniques offer more accurate and rapid results, contributing to improved speed and reliability throughout the entire product lifecycle. By embracing these disruptive innovations, the industry demonstrates its commitment to efficiency, adaptability, and sustainability.

Moving forward, the pharmaceutical industry, in collaboration with academia and regulatory, is actively embracing innovative technologies, sustainable practices, and adaptable manufacturing and testing methodologies. This theme provides a forum for Innovations in small molecule, biologics, cellular therapies, oligonucleotides, and antibody drug conjugates manufacturing and characterization agnostic of their development and implementation stage.

Theme 2: Digital Tools to Accelerate Drug Development, Manufacturing and Analysis

Keywords: Automation and Robotics, Simulation And Modeling Platforms To Increase Efficiency, Virtual Testing And Process Design, Digital Twins, Mini Piloting, Platform Ecosystem For DS and DP, Line of Sight

to Commercialization, Predictive Modeling for Manufacturing, Supply Chain Visibility (Enhanced Traceability And Visibility), Internet Of Things (IOT) Devices, Real-Time Data Collection, Analysis and Decision Making, Training Simulations Using Virtual Reality (VR) and Augmented Reality (AR), Drug Repurposing -- Artificial Intelligence (AI) and Machine Learning (ML) to Identify New Uses, Regulatory Guidance For Digital Tools, Validation Of Digital Tools, Model Maintenance, Safeguards for Digital Tools.

The pharmaceutical industry has wholeheartedly embraced a range of digital tools and platform technologies to expedite drug development, manufacturing, and analysis. These tools harness advanced technologies to enhance efficiency, reduce costs, and improve overall processes. They also leverage historical learning from industry and academia. Key examples of these tools include computational drug design, training simulations, digital twins, cloud computing, high-throughput experiments, crystal structure prediction, regulatory information management systems and standardization of submission data format.

By integrating these tools into the product lifecycle, the pharmaceutical industry can accelerate drug development, manufacturing, and analysis. These tools leverage data automation and advanced analytics to make processes more efficient, cost-effective, and innovative, therefore taking drug to patients faster. They enable the industry to leverage vast amounts of data, leading to faster decision-making and improved outcomes.

The adoption of these tools represents a significant step forward in the pharmaceutical industry's quest for enhanced productivity and competitiveness. The track will also elaborate on the latest in regulatory guidance and safeguards for digital tools.

Theme 3: Challenges and Lessons Learned in Commercializing (Manufacturing and Product Quality Analysis) Novel Modalities

Keywords: Commercialization of Antibody-Drug Conjugates (ADC), Nano-Medicine, Protein Degradation Therapies (PROTACS), Cell Therapy, CRISPER, Bispecifics, Oral Peptide, RNA-Based Therapies, mRNA, SiRNA, Antisense Oligonucleotides, Gene Therapy, Phage Therapy, 3D Printing In Drug Delivery, mRNA Vaccines, Abuse Deterrent Formulations, Life Cycle Management of Analytical Methods, Regulatory Strategy, Intellectual Property Protection, Manufacturing Scalability, Cost of Goods and Manufacturing (COGM) Considerations, Long Term Sustainability and End to End (E2E) Product Lifecycle Management.

Over the past decade, pharmaceutical companies have achieved remarkable success in commercializing novel therapies that have revolutionized patient safety and healthcare outcomes. These innovations include antibody-drug conjugates (ADC), nanomedicine, protein degradation therapies (PROTACS), cell therapy, CRISPER, bispecifics, oral peptide, RNA-based therapies, mRNA, SiRNA, antisense oligonucleotides, gene therapy, phage therapy, 3D printing in drug delivery, mRNA vaccines and abuse deterrent formulations.

These advancements have had a significant impact on improving global health, thanks to the collaborative efforts of industry, academia, and regulators in a rapidly changing environment. Manufacturing facilities have been adapted to meet the unique requirements of these novel modalities, while new analytical characterization methods have been developed, validated, and implemented to better understand these complex therapies and safeguard patients.

Regulators have played a crucial role by providing opportunities for collaboration, offering clear feedback, and providing guidance to ensure the safe and effective development of these innovative therapies. This track aims to highlight success stories, share lessons learned, address challenges, and provide a framework for accelerating innovation while enabling Pharmaceutical Manufacturing 4.0.

Formulation and Delivery

Theme 1: Formulation Approaches to Overcome Development Challenges Across Modalities

Keywords: Modelling, Modality-Specific Formulation Development, Stability and Prediction, Processes and technologies, Quality by Design (QbD).

Accelerated timelines in the drug discovery and development space, combined with increasingly complex drug substances such as biomolecules, RNA-based vaccines, and low-solubility small molecules, are presenting significant challenges to formulation development. This theme will focus on strategies to address and overcome these challenges, including the use of Artificial Intelligence (AI), Machine Learning (ML), and modeling for formulation design and understanding, in-vivo predictive tools, formulation screening tools, risk-based development approaches, overcoming solubility and permeability issues, and improving and predicting formulation performance and stability. Further emphasis will be given to formulation approaches for biologics (e.g., mAbs, ADCs, bispecific antibodies), vaccines (e.g., mRNA, antigen), peptides, chemical entities, gene and cell therapies, highly concentrated formulations for subcutaneous applications, and novel formulations and processes such as additive manufacturing.

Theme 2: Turbocharging Drug Product Development: The Critical Role of Excipients

Keywords: Novel Excipients, Advanced Applications of Traditional Excipients, Material Properties, Physicochemical and Biological Properties, Supply Chain Management, Sustainability, Quality and Regulatory Compliance, Impurities.

Excipients play a pivotal role in the formulation and development of drug products for both chemical substances and biomolecules. Although typically considered pharmacologically inert, excipients influence a wide range of performance characteristics, including manufacturing robustness, stability, and bioavailability. This theme addresses the increasing demand for knowledge about excipients and the regulatory considerations to enable and accelerate the formulation development of new molecular entities, which are growing in complexity. The theme also explores the risk assessment of incorporating novel excipients into drug products such as compatibility and biocompatibility studies. Additionally, the theme encompasses the use of permeation enhancers, complex formulation stabilizers, and lipid and polymeric excipients in various drug delivery techniques. Furthermore, it covers the effects of excipients on the solubility, viscosity, and stability of biologics, such as proteins, mAbs, and ADCs. The discussion

will also include the role of excipients in facilitating the development and administration of high-concentration and high-volume biologics.

Theme 3: Advanced Drug Delivery Technologies for Targeted Therapeutics

Keywords: Targeted Drug Delivery, Controlled Release, Site and Disease-Specific Drug Delivery, Long-Acting Therapeutics, Nanomedicine, Drug-Device Combination, Patient Centricity, Target Product Profile.

Therapeutics that are more specialized and directly target specific disease areas necessitate approaches that extend beyond the scope of traditional drug delivery. Additionally, diverse patient populations and health care settings across different regions pose specific challenges to drug formulation and delivery. This theme covers disease-specific targeting; innovative delivery routes, multifunctional micro- and nano-delivery systems; controlled and modified release; long-acting injectables or implants; and immune-targeted delivery. In addition, use of novel delivery routes and patient-centric dosage forms can be developed. This includes fixed-dose combinations, age-appropriate dosage forms (e.g. pediatric), drug-device combination products (e.g. autoinjectors or patch pumps) enabling patient self-administration; delivery technologies allowing subcutaneous delivery of large volumes; and alternative routes of administration (e.g. intranasal, pulmonary, or transdermal).

Career Development

Theme 1: Elevate your Scientific Career with Strong Business Acumen

In the dynamic world of pharma, possessing a profound understanding of business intricacies is a game-changer for career advancement. Blend your scientific expertise with business savvy to make astute decisions and carve out a thriving career path in the industry. A well-developed technical set of goals coupled with business knowledge will prepare scientists to become leaders in tomorrow's environment.

The following examples are provided to help submitters develop proposal ideas. The Career Development Committee will consider any proposal that fits the track theme and description above.

Example topics:

- Mastering the Art of Contract Negotiation
- Safeguarding Intellectual Property in the Evolving Scientific Landscape
- Unveiling the Secrets in Job Offers: Pharmaceutical Science Career Acceleration
- Securing Resources for Innovations: A Business-Centric Approach for Academic Professionals
- Decoding Stock Options: A Strategic Approach for Pharma Professionals
- Investor Relationship Management: Navigating Objectives Effectively

- Crafting Successful Inter-Company Collaborations in Pharma (In-licensing, Mergers, Acquisitions, Co-developed Assets)
- Entrepreneurship Unleashed: Initiating Your Venture
- Securing Venture Capital for Start-ups: Strategies for Success

Theme 2: Nurturing a Sense of Equity and Inclusion to Build a Diverse Workforce

Globalization has brought pharmaceutical scientists and professionals from diverse cultural, socioeconomic, and educational backgrounds together to achieve common goals and objectives. Successful teams that have benefited from diversity in culture, education, training, and experiences that foster innovative ideas, have promoted and nurtured these values and parameters at the organizational level.

The following examples are provided to help submitters develop proposal ideas. The Career Development Committee will consider any proposal that fits the track theme and description above.

Example topics:

- Cultivating Meaningful Diversity to Enrich a Workforce
- Navigating Unconscious Bias: Strategies for Understanding and Inclusive Decision-making
- Promoting Mental Health and Wellness in a Competitive and Diverse Work Environment
- Effective Communication for Diverse Teams: From Listening to Using Inclusive Languages, to Building a Diverse and Successful Team
- Strategies for Work-life Balance for a Diverse and Successful Team
- Role of Mentorship in Building and Nurturing a Diverse and Successful Workforce
- Creating a safe environment to foster new ideas and innovations.
- Develop Intelligence for Effective Cross-Cultural Collaboration