OCTOBER 15-18, 2024 | WASHINGTON, D.C. & VIRTUAL

THE WATERGATE HOTEL

16TH Annual

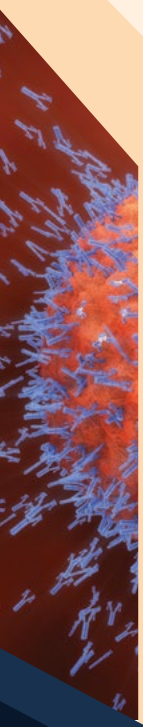
Immunogenicity & Bioassay Summit

Technologies and Strategies for Safe and Efficacious Products in the Clinic

Conference Programs

- Immunogenicity Assessment & Clinical Relevance
- Immunogenicity Prediction & Control
- Optimizing Bioassays for Biologics
- Training Seminar: A Guide to Statistical Methods for Bioassay
- Workshop: Biostatistics for Beginners
- Symposium: Immunology for Biotherapeutics

➤ Plus! 5 Short Courses, Exhibits, Posters, and More



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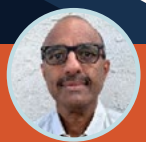
Keynote & Featured Speakers:

Yow-Ming Wang
FDA

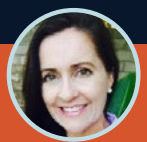


Murli Krishna
Bristol Myers Squibb Co.

Zuben Sauna
FDA

Robert Siegel
Eli Lilly and Company

Kannan Natarajan
NIH



Marisa Joubert
Amgen

Weifeng Xu
Merck

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Join us at the #1 Immunogenicity & Bioassay Event in the U.S.

Bringing Together Industry, Academia & Regulatory Authorities

RESOLVE the CHALLENGES of immunogenicity testing and risk assessment during preclinical and clinical development, bioassay design & analysis, and how to manipulate the immune system for therapeutic advantage by attending CHI's 16th Annual Immunogenicity & Bioassay Summit on October 15-18, 2024, at The Watergate Hotel in Washington, D.C. We'll examine challenges facing the industry with NEW case studies, NEW perspectives, and NEW approaches. Hear from the FDA, industry, academia, and regulatory authorities at 3 Conference Programs, Symposium, Training Seminar, Workshop, and several Short Courses. Once again, we'll host interactive breakouts on various niche topics so delegates can share experiences and generate new ideas. Plus, attend a vibrant exhibit/poster hall with innovative products & services. We hope to greet you this fall in Washington, D.C.

Conference At-a-Glance*

Tuesday, October 15

- **S1:** Immunology for Biotherapeutics
- **SC1:** Development of NAb Assays, Technical Considerations, Case Studies
- **SC2:** Overcoming Drug and Target Interference in ADA and NAb Assays
- **W1:** Biostatistics for Beginners
- **SC3:** Validation of ADA Assays and Cut Point Calculations
- **SC4:** Recent Advances with Cell and Gene Therapy

Wednesday, October 16

- **C1A:** Immunogenicity Assessment & Clinical Relevance
- **TS1A:** A Guide to Statistical Methods for Bioassay

Thursday, October 17

- **C1A:** Immunogenicity Assessment & Clinical Relevance
- **TS1A:** A Guide to Statistical Methods for Bioassay
- **C1B:** Immunogenicity Prediction & Control
- **C2B:** Optimizing Bioassays for Biologics
- **SC5:** Advice on Putting Together an Integrated Summary of Immunogenicity

Friday, October 18

- **C1B:** Immunogenicity Prediction & Control
- **C2B:** Optimizing Bioassays for Biologics

**Short Courses, Workshop & Training Seminar are In-Person Only. See Registration Page for packages and pricing details.*

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CHI offers comprehensive packages that can be customized to your budget and objectives. Sponsorship allows you to achieve your goals before, during, and long after the event. Packages may include presentations, exhibit space, and branding, as well as the use of delegate lists. Signing on early will maximize your exposure to qualified decision-makers and drive traffic to your website in the coming months.

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Showcase your solutions to a guaranteed, targeted audience through a 15- or 30-minute presentation during a specific program, breakfast, lunch, or a pre-conference workshop. Package includes exhibit space, onsite branding, and access to cooperative marketing efforts by CHI. Lunches are delivered to attendees who are already seated in the main session room. Presentations will sell out quickly! Sign on early to secure your talk.

Invitation-Only VIP Dinner/Hospitality Suite

Select specific delegates from the pre-registration list to attend a private function at an upscale restaurant or a reception at the hotel. From extending the invitations, to venue suggestions, CHI will deliver your prospects and help you make the most of this invaluable opportunity.

Focus Group

CHI will gladly provide you the opportunity of running a focus group. This exclusive gathering can be useful to conduct market research, collect feedback on a new product idea, and collect marketing intelligence from industry experts on a specific topic.

User Group Meeting/Custom Event

Co-locate your user group meeting or custom event. CHI will help market the event, manage logistical operations, develop the agenda, and more. CHI can handle the entirety of the meeting or select aspects.

Exhibit

Exhibitors will enjoy facilitated networking opportunities with qualified delegates, making it the perfect platform to launch a new product, collect feedback, and generate new leads. Exhibit space sells out quickly, so reserve yours today! Additional branding and promotional opportunities are available, including:

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- Padfolios and More...

For more information regarding exhibit and sponsorship, please contact:

Aimee Croke

Sr. Business Development Manager
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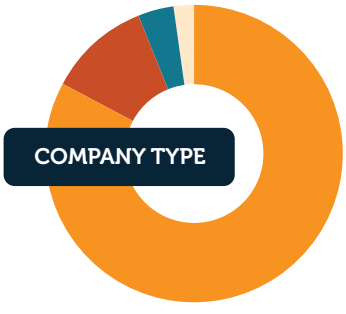
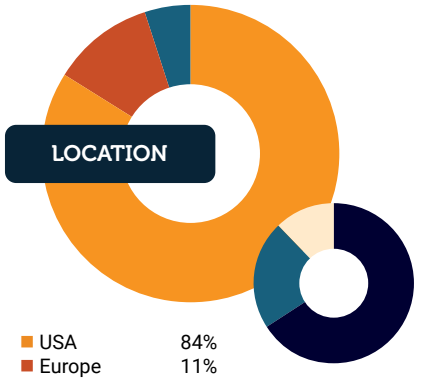
2023 Attendee Demographics





Distinguished Faculty

FDA Speakers

Sojin Bing, PhD, Staff Fellow, Office of Tissues & Advanced Therapies, FDA CBER

Eric Brodsky, MD, Associate Director, Labelling Policy Team, CDER, FDA

Kristina Howard, Principal Investigator, Division of Applied Regulatory Science (DARS), CDER, FDA

Daniel LaGasse, PhD, Research Regulator, CBER, FDA

Ronit Mazor, PhD, Principal Investigator, CBER, FDA

Zuben Sauna, PhD, Director, Division of Hemostasis, CBER, FDA

Anurag Sharma, PhD, Gene Therapy Reviewer, CBER, FDA

Daniela Verthelyi, MD, PhD, Chief, Laboratory of Immunology, CDER, FDA

Leslie Wagner, Consumer Safety Officer, CBER, FDA

Yow-Ming Wang, PhD, Associate Director for Biosimilars and Therapeutic Biologics, CDER, FDA

Additional Distinguished Faculty

Kavitha Akula, PhD, Principal Scientist, Bristol Myers Squibb Co.

Bianca Bautista, PhD, Senior Scientist, Clinical Immunology, Amgen, Inc.

Daron Forman, PhD, Senior Principal Scientist, Discovery Biotherapeutics, Bristol Myers Squibb Co.

Andrew Graves, Director, Immunogenicity Assessment, Specialty Analytics, Teva Pharmaceuticals

Michele Gunsior, PhD, Senior Director, Astria Therapeutics

Kevin Gurney, PhD, Distinguished Scientist, Analytical Research and Development, Merck

Robert Hamilton, PhD, D.ABMLI, Professor, Medicine & Pathology, Clinical Immunology & Allergy, Johns Hopkins University

Timothy Hickling, PhD, Investigative and Immunosafety Chapter, Roche

Andreas Hollenstein, PhD, Principal Scientist, Immunosafety, Roche

Shannon Howell, PhD, Principal Scientist, Immunogenicity Bioanalytical, Amgen, Inc.

Jeffrey A. Hubbell, PhD, Eugene Bell Professor Tissue Engineering & Vice Dean, Molecular Engineering, University of Chicago

Susan Irvin, PhD, Senior Principal Scientist, Bioanalytical Strategy, Regeneron

Nicole Jarvi, PhD, Senior Scientist, Merck

Vibha Jawa, PhD, Executive Director, Nonclinical Disposition and Bioanalysis, Bristol Myers Squibb Co.

Lynn Kamen, PhD, Scientific Officer, Executive Director, BioAgilytix

Emilee Knowlton, Senior Immunology Sales Specialist, ProImmune Inc

Alexander Kozhich, PhD, Director, Bristol Myers Squibb Co.

Murli Krishna, PhD, Senior Principal Scientist, Bristol Myers Squibb Co.

David Lansky, PhD, President, Precision Bioassay, Inc.

Daniel Leventhal, PhD, Head of Immunogenicity, Generate Biomedicines

Linlin Luo, PhD, Senior Director, Merck

Dawn Maier, PHD, Cell and Gene Therapy CMC, AD Preclinical Technical Advisor, DGMAIER Consulting

Laurent P. Malherbe, PhD, Executive Director, Eli Lilly and Company

Arkadi Manukyan, PhD, Senior Scientist, Bioassay Development, Sanofi

Yan Mao, PhD, Senior Principal Scientist, Boehringer Ingelheim Pharmaceuticals

Jim McNally, PhD, CSO, BioAgilytix

Diana Montgomery, PhD, Principal Scientist, Pharmacokinetics, Merck & Co., Inc.

Paul Moore, PhD, CSO, Zymeworks

Kannan Natarajan, PhD, Staff Scientist, NIAID, NIH

Ravish B. Patel, PhD, Senior Manager, Pharmacology, Sun Pharma Advanced Research Center

Ryan Peckner, PhD, Director, Machine Learning, Seismic Therapeutic

Jack A. Ragheb, MD, PhD, Senior Vice President, Translational Sciences and Medicine, NexImmune

Bonnie Rup, PhD, Biotechnology Consultant, Bonnie Rup Consulting

Nancy Sajjadi, Independent Quality Consultant, Sajjadi Consulting

Sumona Sarkar, PhD, Biomedical Engineer, Biosystems and Biomaterials Division, Biomaterials Group, National Institute of Standards and Technology

Han-Yu Shih, PhD, Investigator, NeuroImmune Regulome, National Institutes of Health National Eye Institute

Robert Siegel, PhD, Associate Vice President, Laboratory for Experimental Medicine, Eli Lilly and Company

Matthew Stephenson, PhD, Director of Statistics, Quantics Biostatistics

Michael D. Swanson, PhD, Associate Scientific Director, Bioanalytical Discovery & Development Sciences, Johnson and Johnson Innovative Medicine

Jiangbo Tang, PhD, Senior Scientist, Bristol Myers Squibb Co.

Jenny Valentine, PhD, Senior Principal Scientist, Bioanalytical Sciences, Regeneron

Faye Vazvaei, Executive Director, Merck

Weifeng Xu, PhD, Director, Bioanalytical, Merck

James Zanghi, PhD, Senior Principal Scientist, BioAnalytical Sciences, Genentech, Inc.

An Zhao, PhD, Senior Principal Scientist, Bioanalytical Sciences, Regeneron Pharmaceuticals, Inc.

Short Courses*

**Separate registration is required, see Registration Page for pricing details. Short courses are available in-person only.*

TUESDAY, OCTOBER 15 | 9:00 AM – 12:00 PM

SC1: Development of NAb Assays, Technical Considerations, Case Studies

Instructors:

Lynn Kamen, PhD, Scientific Officer, Executive Director, BioAgilytix

Jim McNally, PhD, CSO, BioAgilytix

The development of neutralizing antibody assays is a daunting task that is complicated by the specific nature of each biotherapeutic. Many factors must be assessed to choose the proper assay format, to develop a robust assay, and choose when to invest in the development and implementation of these assays. This short course will focus on these topics and provide examples of current industry practices and publications.

TUESDAY, OCTOBER 15 | 2:00 PM – 5:00 PM

SC2: Overcoming Drug and Target Interference in ADA and NAb Assays

Instructors:

Lynn Kamen, PhD, Scientific Officer, Executive Director, BioAgilytix

Weifeng Xu, PhD, Director, Bioanalytical, Merck

Soluble drug, drug target, and matrix can often interfere in the detection of anti-drug antibodies, including neutralizing Abs. Although not always straightforward, it can be addressed and mitigated in a properly designed immunoassay. This short course will give an overview of the different types of interferences, and current methodologies and approaches being utilized to resolve or reduce them.

TUESDAY, OCTOBER 15 | 5:30 PM – 8:30 PM

SC3: Validation of ADA Assays and Cut Point Calculations

Instructors:

Kavitha Akula, PhD, Principal Scientist, Bristol Myers Squibb Co.

Krupa Ramani, Manager, Johnson & Johnson

Jiangbo Tang, PhD, Senior Scientist, Bristol Myers Squibb Co.

This short course will focus on the validation of ADA assays and cut-point evaluations. We will provide an in-depth overview of the basic considerations around ADA assay validation, with significant focus on the process of evaluating different types of cut-points, and the translation of the cut-point established during validation to the real-world implementation during a preclinical or clinical study.

TUESDAY, OCTOBER 15 | 5:30 PM – 8:30 PM

SC4: Recent Advances with Cell and Gene Therapy

Instructor:

Jim McNally, PhD, CSO, BioAgilytix

Topics to be covered in this course include: Immunogenicity assessment of cell therapies, examining recent developments with CAR T cells & edited stem cell, immunogenicity assessment of gene therapies, recent data on pre-existing reactivity for AAV, advances with redosing, application of current guidance to novel modalities, and what is your product, the vector, the expressed product?

THURSDAY, OCTOBER 17 | 6:00 PM – 9:00 PM

SC5: Advice on Putting Together an Integrated Summary of Immunogenicity

Instructor:

Bonnie Rup, PhD, Biotechnology Consultant, Bonnie Rup Consulting

The purpose of this workshop is to share experience gained in preparing and reviewing the "Integrated Summary of Immunogenicity (ISI)" for submission in regulatory filings. We will overview examples of the multi-disciplinary information that is most useful for the regulator assessing the scale of risk of undesirable immunogenicity for overall clinical benefit vs. risk. We will also examine the sponsor team's role, the general format of an ISI, and provide examples of how to anticipate and address potential issues (and how to avoid introducing any new ones!) by generating a well-thought-out and constructed integrated summary.

"The Summit was a fantastic meeting. It was an exciting opportunity to connect with colleagues and collaborators and to discuss the cutting-edge developments in protein drug immunogenicity."

- Assistant Professor, The University of Kansas

S1: Immunology for Biotherapeutics

Understanding and Manipulating the Immune System for Therapeutic Advantage

October 15, 2024

TUESDAY, OCTOBER 15

8:30 am Registration and Morning Coffee

CURRENT UNDERSTANDING OF IMMUNE MECHANISMS

9:50 Chairperson's Remarks
Kannan Natarajan, PhD, Staff Scientist, NIAID, NIH

10:00 FEATURED PRESENTATION: Antigen Processing and Presentation: The Basis of T Cell Activation
Kannan Natarajan, PhD, Staff Scientist, NIAID, NIH

Antigen-presenting cells process protein antigens into peptides for binding by either Major Histocompatibility Class I (MHC-I) or Class II (MHC-II) molecules, which are then displayed at the cell surface as peptide/MHC complexes, where they are recognized by T cell receptors leading to T cell activation. Cell biological, biochemical, and structural details of these processes as we now understand them will be discussed.

10:30 Role of IgE and IgG/IgG4 in Modulating Type I Hypersensitivity Reactions in Human Allergic Disease
Robert Hamilton, PhD, D.ABMLI, Professor, Medicine & Pathology, Clinical Immunology & Allergy, Johns Hopkins University

Of the four areas of hypersensitivity, this presentation will focus on immediate Type I IgE-mediated, allergic disease. The presentation examines pathophysiology, current diagnostic strategies, four modes of disease management, and special caveats relating to food, drug, venom, and respiratory allergic disease. Importantly, the newly expanded discipline of molecular allergology will be highlighted with an emphasis on ten cross-reactive allergen families and how allergenic molecules improve the accuracy of allergy diagnosis.

11:00 Networking Coffee Break

11:15 The Role of the Innate Immune System and Implications for Biotherapeutics
Han-Yu Shih, PhD, Investigator, NeuroImmune Regulome, National Institutes of Health National Eye Institute

The field of innate lymphoid cell (ILC) biology has progressed rapidly, highlighting these cells' roles in immunity, barrier tissue integrity, and homeostasis. ILCs can be classified based on cytokine production, mirroring patterns in CD4 T helper (Th) cell analogs. Unlike Th cells, ILCs respond promptly to pathogens without needing antigen-specific receptor recognition. Understanding ILC differentiation and immunoregulation is key to developing new treatments for autoimmunity, infection, and cancer.

11:45 Complement as an Apex Regulator of Tissue Inflammation
Ben Afzali, MD, PhD, FRCP, Earl Stadtman Investigator, Immunoregulation Section, Kidney Diseases Branch, NIDDK, NIH

Our lab investigates tissue inflammation, injury, and repair, focusing on gene expression influenced by micro-environmental signals and transcription factors using functional genomics approaches. An important aspect of our work centers around complement components produced by cells in tissues. In this talk, we will discuss the mechanisms and roles of locally produced complement in inflammation, how this influences tissue biology, and the key transcriptional regulators involved.

12:15 pm Luncheon Presentation *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

12:45 Session Break

HARNESSING THE IMMUNE SYSTEM FOR BIOTHERAPEUTICS

2:00 Chairperson's Remarks
Paul Moore, PhD, CSO, Zymeworks

2:05 Pushing the Boundaries of Antibody-Based Therapeutics through Multispecifics and Drug Conjugates
Paul Moore, PhD, CSO, Zymeworks

Antibody-based therapeutics have provided great therapeutic benefit to many patients across various disease states. Multispecific antibodies afford therapeutic opportunities not feasible with single-target antibodies or combinations, while drug conjugates provide opportunity to extend therapeutic benefit through combining the targeting specificity of an antibody with a "payload." Examples of these advances will be summarized in the context of molecule design, target selection, biological characterization, and clinical benefit.

2:35 Harnessing the Body's Natural Immune Response to Fight Cancer
Daron Forman, PhD, Senior Principal Scientist, Discovery Biotherapeutics, Bristol Myers Squibb Co.

Immunotherapy has shown remarkable response rates in some previously hard-to-treat cancers by redirecting the body's own immune system to recognize and eliminate tumor cells. Here, we will discuss the current state of immunotherapy by briefly covering cytokine therapy, cancer vaccines, adoptive immunotherapy, and immunomodulation therapy.

3:05 Networking Refreshment Break

3:30 Emerging Immunogenicity Obstacles for Next-Generation Biotherapeutics
Andreas Hollenstein, PhD, Principal Scientist, Immunosafety, Roche

Since the beginning of immunotherapy using monoclonal antibodies, we have pushed for higher efficiency of this therapeutic modality. This shift did not come without trade-offs for safety and immunogenicity. This presentation will cover several examples of drug-enhancing approaches that also can have a profound impact on immunogenicity. Understanding the underlying mechanisms could help to stay far away from immunogenic transformation and lead to the development of better drugs for patients.

4:00 PANEL DISCUSSION: Harnessing the Immune System for Biotherapeutics
Moderator: Paul Moore, PhD, CSO, Zymeworks
Panelists:
Daron Forman, PhD, Senior Principal Scientist, Discovery Biotherapeutics, Bristol Myers Squibb Co.
Andreas Hollenstein, PhD, Principal Scientist, Immunosafety, Roche

4:30 Close of Immunology for Biotherapeutics Symposium

5:00 Dinner Short Course Registration

5:30 Recommended Dinner Short Course*
SC4: Recent Advances with Cell and Gene Therapy
*Separate registration required. See short course page for details.

THURSDAY, OCTOBER 17

6:00 pm Recommended Dinner Short Course*
SC5: Advice on Putting Together an Integrated Summary of Immunogenicity
*Separate registration required. See short course page for details.

W1: Biostatistics for Beginners*

October 15, 2024

**Available in-person only*

TUESDAY, OCTOBER 15 | 9:00 AM – 5:00 PM

W1: Biostatistics for Beginners

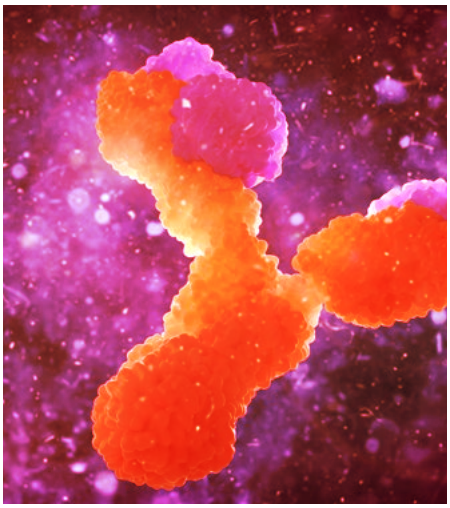
Instructor:

Nancy Sajjadi, Independent Quality Consultant, Sajjadi Consulting

Statistical analysis is an integral part of designing, developing, validating, and implementing a bioassay. Statistical tools and techniques are required for experimental design, selecting an appropriate model for the dose response curve, evaluating system and sample suitability, and to measure, control, and communicate the uncertainty of reported potency results. Fundamental concepts are also integral to developing product specifications and making decisions for lot release. This workshop is intended for people who generate or review bioassay data but have minimal training in statistics. This slower-paced course will help participants understand the meaning of commonly encountered statistical information in context and expand their knowledge of fundamental concepts and tools that are applicable to their daily work. Sufficient time is allocated at the end of the day to review specific topics by request and further discuss participant questions.

Nancy Sajjadi, Independent Quality Consultant, Sajjadi Consulting

Nancy Sajjadi, M.Sc. is Founder and Principal Consultant of Sajjadi Consulting. She has over 30 years of experience in biopharmaceutical product development. She began her career as a bench scientist doing malaria vaccine research then joined a start-up company developing cell and gene using therapies for infectious disease, cancer and cell therapy applications. Her responsibilities included research, assay development, and quality control. In 2000, she left her position as Director of QC at Chiron Technologies Center for Gene Therapy to start her own consulting business. For over 20 years, Ms. Sajjadi has provided services to biopharmaceutical companies, contract laboratories, non-profit organizations, universities and US government agencies. She has assisted clients in the development, implementation and improvement of quality programs for a range of cutting-edge products and provides technical expertise in assay development, qualification and validation. Ms. Sajjadi has authored several articles pertaining to bioassays and viral gene therapy and has served on 5 advisory panels for the United States Pharmacopeia (USP). She enjoys teaching introductory courses in bioassay design, development and validation for non-statisticians. Her company has recently expanded services to include leadership development and executive coaching to guide organizations toward sustaining a culture of quality.



"A great opportunity to meet FDA colleagues and interact with experts in different fields."

- Bioanalytical Principal Investigator, Senior Scientist, Pfizer

C1A: Immunogenicity Assessment & Clinical Relevance

Assay Strategy for Meaningful Evaluation

TUESDAY, OCTOBER 15

9:00 am Recommended Short Course*
SC1: Development of NAb Assays, Technical Considerations, Case Studies
*Separate registration required. See short course page for details.

2:00 pm Recommended Short Course*
SC3: Validation of ADA Assays and Cut Point Calculations
*Separate registration required. See short course page for details.

5:30 Recommended Dinner Short Course*
SC5: Advice on Putting Together an Integrated Summary of Immunogenicity
*Separate registration required. See short course page for details.

WEDNESDAY, OCTOBER 16

7:45 am Registration and Morning Coffee

CLINICAL RELEVANCE OF IMMUNOGENICITY—LATEST FDA FEEDBACK

8:25 Chairperson's Opening Remarks
Alexander Kozhich, PhD, Director, Bristol Myers Squibb Co.

8:30 KEYNOTE PRESENTATION: The Role of Clinical Pharmacology and Clinical Relevance of Immunogenicity
Yow-Ming Wang, PhD, Associate Director for Biosimilars and Therapeutic Biologics, CDER, FDA

To address the concern of negative impact of immunogenicity on treatment effects, therapeutic drug monitoring has been adopted as a mechanism of patient management in some clinical specialties. Advances in leveraging clinical pharmacology data have offered an option for evaluating clinical impacts. This presentation will discuss the current challenges and potential future directions based on the review experience in the Office of Clinical Pharmacology at the FDA.

9:00 Therapeutic Drug Monitoring for Biologics—Challenges and Opportunities
Michele Gunsior, PhD, Senior Director, Astria Therapeutics

Therapeutic drug monitoring (TDM) for large molecules is not widely practiced despite reported benefits in patient outcomes for some immune-mediated inflammatory diseases. A joint US-FDA (OCP)/AAPS workshop was convened in February 2024, to discuss the potential benefits, limitations, and future considerations for implementation of large molecule TDM. Among the potential benefits is preventing secondary loss of response due to suboptimal dosing and potential generation of anti-drug antibodies.

9:30 Rational ≠ Radical: A Scientific Perspective on Immunogenicity Risk and Assessment
Lauren Stevenson, CSO & Head of Translational Sciences, Immunologix Labs

This presentation will highlight the context in which current practices for immunogenicity risk assessment and the 3-tiered testing paradigm were developed, followed by discussion of lessons learned over the last 20+ years of implementation. Leveraging those learnings, a scientific perspective that reframes immunogenicity as a biomarker will be presented that demonstrates how adopting a biomarker mindset with focus on context-of-use can be applied to (1) refine risk assessments to focus on likelihood of clinically meaningful immunogenicity (2) develop assay and testing strategies that enable a more comprehensive view of immunogenicity data, and (3) bring clarity to data interpretation that simplifies communication of conclusions to key stakeholders, with potential to reduce burden on regulatory reviewers.

10:00 Networking Coffee Break

10:15 Challenges Associated with Assessing the Clinical Effects of Immunogenicity
Diana Montgomery, PhD, Principal Scientist, Pharmacokinetics, Merck & Co., Inc.

Evaluating the clinical effects of immunogenicity has become more challenging with the advent of highly sensitive ADA assays that result in a higher proportion of ADA-positive participants. This talk will discuss the challenges associated with constructing a cross-functional group to investigate potential clinical effects on PK, PD, efficacy, and safety. Displays helpful for exploration of the data and in distilling meaningful clinical effects will be presented.

FDA UPDATES - LABELING, PEPTIDES, ASSESSMENT

10:45 Guidance on Immunogenicity in Drug Product Labeling
Eric Brodsky, MD, Associate Director, Labelling Policy Team, CDER, FDA

This presentation will discuss incorporating immunogenicity information into the prescribing information for human drugs and biological products.

11:05 Presentation to be Announced

11:25 Assessing Immunogenicity Using Immune Humanized Mice
Kristina Howard, Principal Investigator, Division of Applied Regulatory Science (DARS), CDER, FDA

This talk discusses the use of immune humanized mice to assess immunogenicity in drug development. It explores the methodology for creating and utilizing these mice to mimic human immune responses, providing a valuable preclinical tool for predicting immunogenic reactions to therapeutics. The focus is on enhancing the accuracy of immunogenicity assessments, which is crucial for the safety and efficacy of new drugs, particularly biologics and biosimilars.

11:45 Luncheon Presentation *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

12:15 pm Session Break

IMMUNOGENICITY OF GENE THERAPIES AND AAVs

1:15 Chairperson's Remarks
Ronit Mazor, PhD, Principal Investigator, CBER, FDA

1:20 Immunogenicity Assessment of Gene Therapy Products
Anurag Sharma, PhD, Gene Therapy Reviewer, CBER, FDA

Despite great promise, the immunogenicity of gene therapies remains one of the biggest challenges. Immunogenicity can impact the safety, efficacy, and long-term durability of gene therapies. Addressing immunogenicity-associated challenges is critical to realizing the full potential of these life-saving therapies. In my talk, I will discuss FDA's perspective on the immunogenicity associated with gene therapies.

1:50 Rational Immunosilencing of a Promiscuous T Cell Epitope in the Capsid of an Adeno-Associated Virus
Sojin Bing, PhD, Staff Fellow, Office of Tissues & Advanced Therapies, FDA CBER

We aim to reduce immune responses to AAV therapeutics by elimination of capsid T cell epitopes within the AAV capsid. To reduce or eliminate immunogenicity, it is vital that the method that eliminates MHC-binding epitopes does not disrupt vector structure and function. Here, we describe the rationally designed chimeric AAV vector using integration of *ex vivo* T cell assays, MHC epitope prediction, and sequence conservation analysis in AAV phylogeny.

INTERACTIVE DISCUSSIONS: IN-PERSON ONLY

2:20 Interactive Discussions
Interactive Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

TABLE 2: Recommendations for Harmonization of Neutralizing ADA Testing and Reporting
Michele Gunsior, PhD, Senior Director, Astria Therapeutics

TABLE 3: When One Size Does Not Fit All: Reevaluating Immunogenicity Risk Assessments and The 3-Tiered Testing Paradigm
Lauren F. Stevenson, PhD, CSO & Head, Translational Sciences, Immunologix Labs

3:10 Grand Opening Refreshment Break in the Exhibit Hall with Poster Viewing

IMMUNOGENICITY OF NOVEL THERAPIES, CAR Ts

4:00 Immunogenicity Strategy and Challenges for Newer Modalities
Jenny Valentine, PhD, Senior Principal Scientist, Bioanalytical Sciences, Regeneron
Many drug modalities in clinical development are significantly more complex than traditional monoclonal antibodies. While the ADA assessment strategy for mAbs is well-established in White Papers and regulatory guidance, the immunogenicity evaluation requirements for newer modalities are still largely undefined. In this presentation we will discuss several newer modalities and the immunogenicity challenges for ADA/NAb testing, focusing on how the overall immunogenicity assessment will be used to inform clinical decisions.

4:30 FEATURED PRESENTATION: Immunogenicity to CAR T Therapy: Mechanisms, Clinical Considerations, and Mitigation Strategies
Murli Krishna, PhD, Senior Principal Scientist, Bristol Myers Squibb Co.
Immunogenicity of CAR T cell therapy can influence effectiveness and lead to potential side effects. This presentation will discuss factors contributing to the immunogenicity, studies that demonstrate its impact on CAR T cell therapy outcomes like persistence and functionality of CAR T cells, and CAR design technologies for mitigating it.

5:00 Blood Processing Considerations for Clinical Cellular Immunogenicity Assessment
Michael D. Swanson, PhD, Associate Scientific Director, Bioanalytical Discovery & Development Sciences, Johnson and Johnson Innovative Medicine
Cellular immune responses have the potential to impact clinical safety and efficacy of various modalities including gene, cellular, and protein-based therapies. In this presentation, we will discuss how variables such as patient blood draw, transit conditions, peripheral blood mononuclear cell isolation, and storage can impact downstream assays that assess cellular immune responses to therapeutics.

5:30 Welcome Reception in the Exhibit Hall with Poster Viewing

6:30 Close of Day

THURSDAY, OCTOBER 17

7:30 am Registration and Morning Coffee

ANALYSIS OF IMMUNOGENICITY

7:55 Chairperson's Remarks
Susan Irvin, PhD, Senior Principal Scientist, Bioanalytical Strategy, Regeneron

8:00 The Immunogenicity Journey towards Winrevair FDA Approval
Linlin Luo, PhD, Senior Director, Merck
With the recent approval for Winrevair, we believe it is valuable to share Merck's experience in immunogenicity data analysis and reporting, as well as our approaches for analyzing the clinical impact of immunogenicity. These efforts have culminated in the development of the Integrated Summary of Immunogenicity (ISI) contributing to FDA approval for Winrevair.

8:30 Analysis of Clinical Immunogenicity Data for a Multi-Study Program
Susan Irvin, PhD, Senior Principal Scientist, Bioanalytical Strategy, Regeneron
We report data from a mAb therapeutic across multiple studies that include an examination of the impact of dose, route of administration, sample time points, and patient populations. We highlight the relationships among these aforementioned variables, as well as important implications for development of therapeutic proteins in the future.

9:00 Presentation to be Announced
Cerba Research

9:30 Coffee Break in the Exhibit Hall with Poster Viewing

10:15 Intravenous vs. Subcutaneous: How Delivery Route Impacts Immunogenicity of Protein Therapeutics
An Zhao, PhD, Senior Principal Scientist, Bioanalytical Sciences, Regeneron Pharmaceuticals, Inc.
Subcutaneous delivery of therapeutic proteins can increase patient compliance and reduce burden on healthcare systems compared to intravenous infusion. However, subcutaneous administration has been proposed to increase immunogenicity. We reviewed anti-drug antibody (ADA) data for various protein therapeutics that were administered both intravenously and subcutaneously, and overall, no difference in immunogenicity incidence was observed. Although administration route may in some instances influence ADA, other risk factors are likely more impactful.

10:45 Detection and Characterization of Preexisting Antidrug Antibodies towards Multi-Domain Biotherapeutics—A Case Study
Yan Mao, PhD, Senior Principal Scientist, Boehringer Ingelheim Pharmaceuticals
This presentation will focus on a case study from one of Boehringer-Ingelheim's multi-domain biotherapeutic programs to demonstrate our strategies for detection and characterization of PE-ADA. This case will provide in detail the specialized tool molecules along with the methods used to characterize the domain specificity and identify the location of PE-ADA. It will also share our strategy for the development of an ADA method to distinguish between PE-ADA and TE-ADA.

11:15 Revisiting Antidrug Antibody Assay Drug Tolerance
James Zanghi, PhD, Senior Principal Scientist, BioAnalytical Sciences, Genentech, Inc.
Drug tolerance is a critical attribute of anti-drug antibody (ADA) assays for assessing clinical immunogenicity. We present a case study where a previously approved product required reassessment of the assay drug tolerance to meet both increased drug exposure demands and more rigorous validation requirements since the product's launch. The strategy to overcome these issues while working within challenging timelines can be broadly applied to other therapeutics.

11:45 Close of Immunogenicity Assessment & Clinical Relevance Conference

6:00 pm Recommended Dinner Short Course*
SC5: Advice on Putting Together an Integrated Summary of Immunogenicity
*Separate registration required. See short course page for details.

C1B: Immunogenicity Prediction & Control

Regulatory Perspectives, Risk Management, and Predictive Tools

TUESDAY, OCTOBER 15

9:00 am Recommended Short Course*
SC1: Development of NAb Assays, Technical Considerations, Case Studies
*Separate registration required. See short course page for details.

2:00 pm Recommended Short Course*
SC4: Recent Advances with Cell and Gene Therapy
*Separate registration required. See short course page for details.

5:30 Recommended Dinner Short Course*
SC5: Advice on Putting Together an Integrated Summary of Immunogenicity
*Separate registration required. See short course page for details.

THURSDAY, OCTOBER 17

12:00 pm Registration Open

IMMUNOGENICITY RISK ASSESSMENT—mAbs to ADCs

1:15 Chairperson's Remarks
Jack A. Ragheb, MD, PhD, Senior Vice President, Translational Sciences and Medicine, NexImmune

1:20 Immunogenicity Risk Assessment and Mitigation
Timothy Hickling, PhD, Investigative and Immunosafety Chapter, Roche
Immunogenicity risk assessments can be used to optimize portfolios to acceptable immunogenicity risk levels. ADA responses vary widely in clinical trials, with the presence of ADA itself a risk factor for clinical impact. Whilst 'predicting immunogenicity' might seem impossible, forecasting incidence of ADA formation in first-in-human trials helps to prepare teams for potential consequences. I review a risk assessment methodology, highlighting case studies to demonstrate application at the portfolio level.

1:50 Immunogenicity of the Next-Generation of Antibody-Drug Conjugates
Alexander Kozhich, PhD, Director, Bristol Myers Squibb Co.
Over the last several years there has been exponential increase of antibody-drug conjugates in various stages of development. Several novel ADC formats being explored include unnatural antibodies (bispecifics, probodies, etc.) as well as novel payloads (immune-stimulators, protein degraders, etc.). It is important to understand the immunogenicity risk of these novel ADCs and their clinical relevance. Better understanding of potential immunogenicity of these novel ADCs should help in their clinical development.

2:20 Preclinical Risk Assessment
Jack A. Ragheb, MD, PhD, Senior Vice President, Translational Sciences and Medicine, NexImmune
Testing of biotherapeutics across species typically induces an Anti-Drug Antibody (ADA) response. When the therapeutic is a monoclonal antibody (mAb), these ADA are generally directed against the constant regions of the mAb, but may be against other epitopes. The impact of these ADA on PK, PD, and the toxicity profile of the therapeutic mAb, as well as novel ways to mitigate their formation, will be examined.

2:50 An Integrated Approach to Managing Immunogenicity Risk and Optimum Protein Design
PROIMMUNE www.proimmune.com
Andrew Isidoridy, Immunology Sales Specialist, ProImmune, Inc.
Immunogenicity risk assessment is an essential step in bringing therapeutic drugs to the market. ProImmune's risk management tools evaluate immunogenic epitopes and the corresponding functional T cell responses that can lead to unwanted immune responses. Case studies will highlight how the integrated platform is used to address key questions in the drug development phase.

3:20 Refreshment Break in the Exhibit Hall with Poster Viewing

4:00 KEYNOTE PRESENTATION: What Information Can MHC Associated Peptide Proteomics (MAPPs) Assays Provide during Immunogenicity Risk Assessment of Therapeutic Proteins and Novel Modalities?
Zuben Sauna, PhD, Director, Division of Hemostasis, CBER, FDA
In vitro assays that assess the potential immunogenicity of protein therapeutics are useful during drug development. I will provide specific examples of the application of MAPPs in assessing the immunogenicity of protein therapeutics (Factor VIIa and Factor VIII). I will also discuss MAPPs assays in the context of novel modalities. Many novel modalities rely on intracellular protein synthesis. Novel workflows for MAPPs assays to capture these events will be provided.

4:30 FEATURED PRESENTATION: Evolution of the MAPPs Assay: Gaining Greater Resolution for Populations at Risk for ADA
Robert Siegel, PhD, Associate Vice President, Laboratory for Experimental Medicine, Eli Lilly and Company
A novel B cell MAPPs platform suitable for the examination of a variety of therapeutic antibody scaffolds has been developed. The sensitivity and reproducibility of the system was determined. Most importantly, experiments were performed to assess the similarity of results to those obtained from the traditional monocyte-derived dendritic cell approach. Observations regarding the modification state of cysteine residues involved in MHC-binding core will also be discussed.

5:00 HESI/AAPS: Towards Harmonization of *in vitro* T Cell Assays to Predict Immunogenicity of Biologics
Laurent P. Malherbe, PhD, Executive Director, Eli Lilly and Company
In vitro assays are widely used preclinically during the development of biologics to assess immunogenicity liabilities and select candidates. However, there is considerable diversity in assay format among companies and no alignment on the analysis methods. Here we will discuss the recent progress of a cross-industry work to develop common reference biologics for preclinical immunogenicity risk assessment, a critical first step toward assay standardization and harmonization.

5:30 Close of Day

5:30 Dinner Short Course Registration

6:00 Recommended Dinner Short Course*
SC5: Advice on Putting Together an Integrated Summary of Immunogenicity
*Separate registration required. See short course page for details.

FRIDAY, OCTOBER 18

8:00 am Registration and Morning Coffee

UNDERSTANDING AND CIRCUMVENTING THE IMMUNE SYSTEM

8:25 Chairperson's Remarks
Laurent P. Malherbe, PhD, Executive Director, Eli Lilly and Company

8:30 Understanding and Circumventing the Immune Responses to Approved Protein Therapeutics
Daniel LaGasse, PhD, Research Regulator, CBER, FDA
Immunogenicity can compromise the safety and/or efficacy of therapeutic protein products, and is a priority issue for regulatory agencies. In addition to poor patient outcomes, the social and economic costs associated with neutralizing antibodies are considerable. In this presentation, I survey the immunogenicity of approved therapeutic proteins, discuss strategies for clinical management of immunogenicity, and identify challenges associated with circumventing the immune responses to approved protein therapeutics.

9:00 Presentation to be Announced

Lonza

INTERACTIVE DISCUSSIONS: IN-PERSON ONLY

9:30 Interactive Discussions
Interactive Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

TABLE 1: HESI/AAPS: Towards Harmonization of *in vitro* T Cell Assays to Predict Immunogenicity of Biologics
Laurent P. Malherbe, PhD, Executive Director, Eli Lilly and Company

TABLE 2: End-to-End Immunogenicity: Risk Assessment and Mitigation
Timothy Hickling, PhD, Investigative and Immunosafety Chapter, Roche

TABLE 3: Immunogenicity of Next-Generation Modalities, Including Antibody-Drug Conjugates
Alexander Kozhich, PhD, Director, Bristol Myers Squibb Co.

10:20 Coffee Break in the Exhibit Hall & Last Chance for Poster Viewing

RISK ASSESSMENT AND MANAGEMENT

11:00 FEATURED PRESENTATION: Innate Immune Response Modulating Impurities Testing for Immunogenicity Risk Assessments
Daniela Verthelyi, MD, PhD, Chief, Laboratory of Immunology, CDER, FDA

Comparative *in vitro* analytical methods to characterize innate immune response modulating impurities can inform the immunogenicity risk and help streamline their development of generic peptides and biosimilar proteins. This talk will discuss the risk posed by innate immune response modulating impurities to product immunogenicity, available assays and data interpretation, as well as common pitfalls and remaining knowledge gaps.

11:30 Application of *in vitro* Immunogenicity Assays for Generic Peptide ANDA Submissions
Andrew Graves, Director, Immunogenicity Assessment, Specialty Analytics, Teva Pharmaceuticals

In vitro assays are a requirement for characterizing the immunogenicity risk of impurities in generic peptide candidates submitted for ANDA consideration to the FDA in lieu of clinical immunogenicity assessment. Guidance issued in 2021 has led to a maturing of the science and assays supporting these ANDA submissions today. Here, we review an industry perspective on applying these assays for generic peptides and possibly for other classes of drugs.

12:00 pm Evaluating Pre-Existing Reactivity in Biotherapeutic Development
Shannon Howell, PhD, Principal Scientist, Immunogenicity Bioanalytical, Amgen, Inc.

As the landscape of biologic therapeutics continues to evolve with new and intricate innovations, it is imperative to conduct a thorough assessment of immunogenicity risks and devise a clinical strategy to mitigate potential immunological adverse events. An integral component of this risk assessment involves evaluating the presence of pre-existing antibodies (pre-ADAs). This presentation will introduce a multi-platform approach for detecting and characterizing pre-ADAs, complemented by a detailed case study.

12:30 Luncheon Presentation *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

1:00 Session Break

1:30 Chairperson's Remarks
Sofie Pattijn, Founder & CTO, ImmunXperts, a Q2 Solutions Company

1:35 A Novel B Cell-CD4 T Cell Co-Culture Assay for Preclinical Immunogenicity Risk Assessment
Bianca Bautista, PhD, Senior Scientist, Clinical Immunology, Amgen, Inc.

Effective preclinical prediction of immunogenicity risk is an ongoing challenge for biologics development. A key driver of a clinically impactful anti-drug antibody response is the interaction between antigen-specific B cells and CD4 T cells in the germinal center. We developed a novel B-T cell co-culture assay that can flag molecules with both T and B cell epitopes, identifying those most likely to drive a clinically impactful immune response.

DEIMMUNIZATION, DEVELOPABILITY, AND MACHINE LEARNING

2:05 Accurate HLA Antigen Presentation Prediction Across all HLA-II Loci and its Application for Protein Immunogenicity Assessment
Morten Nielsen, Professor, PhD, Department of Health Technology, Technical University of Denmark

HLA antigen presentation is the cornerstone of adaptive immunity. My talk will describe our journey towards accurate prediction of HLA antigen presentation. I will demonstrate how the end result has unprecedented accuracy across all three HLA-II loci, and how this reveals an earlier underappreciated contribution of DRB345, DQ and DP to the immunopeptidome. Further, I will showcase how these loci share a critical role when interpreting MAPPs data and predicting immunogenicity.

2:35 FEATURED PRESENTATION: Glycotargeting as Induction to Reduce Antidrug Immune Responses
Jeffrey A. Hubbell, PhD, Eugene Bell Professor Tissue Engineering & Vice Dean, Molecular Engineering, University of Chicago

The liver plays key roles in maintenance of peripheral tolerance, for example, clearing and processing apoptotic debris including post-translational modifications that are not centrally tolerized. We have developed glycopolymer conjugation approaches to exploit the same antigen processing pathways utilized in the above, but for protein drugs. Administration of the glycoconjugates in an induction regimen reduces generation of anti-drug antibodies via modulation of T cell help.

3:05 From De-Immunization to Developability: Elucidating the Principles behind the Uptake of Antibodies by Antigen Presenting Cells
Daniel Leventhal, PhD, Head, Immunogenicity, Xaira Therapeutics

Immunogenicity mitigation is a multifactorial design challenge. As a proof-of-concept study, we sought to reduce MHC-II presentation of an immunogenic monoclonal antibody. While T cell antigenicity was reduced, this correlated with improvements in developability (self-association and polyspecificity) rather than elimination of MHC II epitopes. This prompted an expanded analysis of over 100 clinically evaluated antibodies that confirmed a correlation between self-association and polyspecificity with dendritic cell uptake and immunogenicity risk.

3:35 Machine Learning for Deimmunization and Multi-Objective Optimization of Biologics
Ryan Peckner, PhD, Director, Machine Learning, Seismic Therapeutic

We develop machine learning models to optimize in parallel multiple drug-like properties of biologics while minimizing liabilities including immunogenicity. We apply these to the bacterial enzyme IdeS to design a therapeutic for chronic autoantibody-mediated diseases, and demonstrate the success of this approach via *in vivo* and *in vitro* assays. We also illustrate its generalizability by applying it to deimmunize a wide array of clinically approved biologics *in silico*.

4:05 Close of Summit

C2B: Optimizing Bioassays for Biologics

Successful Bioassay Development in an Era of Emerging Therapeutic Modalities

TUESDAY, OCTOBER 15

9:00 am Recommended Short Course*
SC1: Development of NAb Assays, Technical Considerations, Case Studies
*Separate registration required. See short course page for details.

2:00 pm Recommended Short Course*
SC2: Overcoming Drug and Target Interference in ADA and NAb Assays
*Separate registration required. See short course page for details.

5:30 Recommended Dinner Short Course*
SC3: Validation of ADA Assays and Cut Point Calculations
*Separate registration required. See short course page for details.

THURSDAY, OCTOBER 17

12:00 pm Registration Open

BIOASSAY DESIGN AND POTENCY ASSAY DEVELOPMENT

1:15 Chairperson's Remarks

Ravish B. Patel, PhD, Senior Manager, Pharmacology, Sun Pharma Advanced Research Center

1:20 Impact of Product Variants on Product Functional Activity and Stability in the Context of the Biosimilars

Ravish B. Patel, PhD, Senior Manager, Pharmacology, Sun Pharma Advanced Research Center

A potency assay is a regulatory requirement for the release of every lot of a biotherapeutic. For complex biologics, manufacturing processes may be optimized to yield an active ingredient, but a company may end up with reduced activity due to removal of key components. Changes in potency can occur through a variety of physicochemical or structural changes. A meaningful and reliable assay is the most important tool in drug development.

1:50 Limiting Potency Bias from Allowed Non-Similarity While Protecting the Similarity Pass Rate

David Lansky, PhD, President, Precision Bioassay, Inc.

Protecting bioassay-based estimates of relative potency against bias and unacceptably high similarity failure rates, while allowing for changes in assay capability appears to be impractical. While power calculations for both detecting non-similarity (via difference tests) and for passing similar samples (via equivalence tests) are helpful, these calculations are not simple. This presentation will illustrate tools for these calculations and how they support modifying similarity criteria based on data from bioassay.

2:20 Simplifying Cell-Based Potency Assays for Biologics and Gene Therapy Products

Arkadi Manukyan, PhD, Senior Scientist, Bioassay Development, Sanofi

Potency assays are essential to demonstrate the mechanism-of-action of drug products in clinical trials for regulatory approval. Here, we discuss the strategies and challenges of the development and qualification of potency assays for gene therapy products and biologics. Two case studies will be used to demonstrate how to simplify the method by making it more robust, less time-consuming, easy to maintain, and requiring fewer critical reagents.

2:50 Title: Unlocking the Potential of Bioanalytical Automation: Bridging the Gap between Development and Application

Tom Zhang, Chief Scientist, Large Molecule Bioanalysis, www.worldwide.com

Introduction to Bioanalytical Automation: Overview of the development of bioanalytical automation over the years. Key technological advancements and innovations in the field.
Current State of Application: Examination of the limited application of automation in bioanalytical laboratories. Comparison with other fields where automation is extensively applied.
Challenges and Barriers: Identification of major obstacles preventing widespread adoption in bioanalysis. Discussion on technical, regulatory, and operational challenges.
Benefits of Automation: Potential improvements in efficiency, accuracy, and reproducibility. Impact on data integrity, compliance, and throughput.
Case Studies and Success Stories: Examples of successful implementation of automation in bioanalytical settings. Lessons learned and best practices from these implementations.
Future Prospects: Emerging trends and future directions for bioanalytical automation. Potential for integration with AI and machine learning for enhanced capabilities.

3:20 Refreshment Break in the Exhibit Hall with Poster Viewing

4:00 Novel Cell Construct Design for Development of a Functional Cell-Based Potency Assay

Kevin Gurney, PhD, Distinguished Scientist, Analytical Research and Development, Merck

An immunotherapy that binds to ILT3 can block the ligand-ILT3 interaction, reversing an inhibitory signal cascade. A novel chimeric receptor reporter cell-line was designed and engineered that inverted ILT3 from an inhibitory to an activation motif, ITIM to ITAM to measure drug-induced regulation of gene transcription. This cell-based potency assay enabled anti-ILT3 drug disruption of ligand interaction and was sensitive to anti-ILT3 drug stability stresses.

4:30 Considerations for Potency Assurance of CGT Products

Diana Colleluori, PhD, MBA, Principal CMC Consultant, CMC Analytical, Biologics Consulting Group

Sponsors must develop a potency assurance strategy early in drug product development. The recently issued FDA draft guidance on potency assurance of CGT products will be discussed, along with a risk-based approach to developing a product potency assurance and bioassay development strategy.

STANDARDS AND REFERENCE MATERIALS

5:00 Standards Development and Measurement Assurance Strategies for Cell Characterization Assays

Sumona Sarkar, PhD, Biomedical Engineer, Biosystems and Biomaterials Division, Biomaterials Group, National Institute of Standards and Technology

The manufacturing and release of cellular therapy products requires high-quality, robust, and validated analytical methods. Here I will describe recent developments in analytical method standardization, including the FDA Standards Recognition Program and NIST public-private partnerships to support the development of critical analytical methods. A key aspect of analytical development is the need for a fit-for-purpose approach. Efforts to identify and establish fit-for-purpose process analytical technologies will also be described.

5:30 Close of Day

5:30 Dinner Short Course Registration

6:00 Recommended Dinner Short Course*
SC5: Advice on Putting Together an Integrated Summary of Immunogenicity
*Separate registration required. See short course page for details.

FRIDAY, OCTOBER 18

8:00 am Registration and Morning Coffee

GETTING DOWN TO BIOASSAY BRASS TACKS: MEASURING POTENCY-RELATED CRITICAL QUALITY ATTRIBUTES FOR CELL AND GENE THERAPY (CGT) PRODUCTS

8:25 Chairperson's Remarks

Nancy Sajjadi, Independent Quality Consultant, Sajjadi Consulting

8:30 Biologist and Biostatistician Regulatory Perspectives on

Development of Bioassays to Support CGT Products

Jennifer Kirk, PhD, Lead Mathematical Statistician, FDA CBER
Leslie Wagner, Consumer Safety Officer, FDA CBER

9:00 Presentation to be Announced

9:15 Presentation to be Announced *(Opportunity Available)*

INTERACTIVE DISCUSSIONS: IN-PERSON ONLY

9:30 Interactive Discussions

Interactive Discussions are informal, moderated discussions, allowing participants to exchange ideas and experiences and develop future collaborations around a focused topic. Each discussion will be led by a facilitator who keeps the discussion on track and the group engaged. To get the most out of this format, please come prepared to share examples from your work, be a part of a collective, problem-solving session, and participate in active idea sharing. Please visit the Breakout Discussions page on the conference website for a complete listing of topics and descriptions.

TABLE 4: Potency Assays for Personalized Therapeutics Like CAR T Cells

Dawn Maier, PhD, Cell and Gene Therapy CMC, AD Preclinical Technical Advisor, DGMAIER Consulting

- What are the types of biological assays available?
- What are the challenges associated with those assays?
- Why is potency for personalized therapy more complex than traditional biologics?
- What are potential surrogate assays?

TABLE 5: The Impact of Glycosylation on the Functional Activity of Therapeutic Proteins

Ravish B. Patel, PhD, Senior Manager, Pharmacology, Sun Pharma Advanced Research Center

10:20 Coffee Break in the Exhibit Hall & Last Chance for Poster Viewing

11:00 The Challenges Associated with Developing a Potency Assay for Advanced Therapies Such as CAR T Cells and the Importance of Qualifying Those Assays Early in Clinical Development—Lessons Learned

Dawn Maier, PhD, Cell and Gene Therapy CMC, AD Preclinical Technical Advisor, DGMAIER Consulting

Advanced therapies such as CAR T cells are complex living drugs with multiple mechanisms-of-action. Due to this complexity, the strategy for developing and qualifying a single potency assay that correlates with efficacy has been challenging. In this talk, we will address the pros and cons of various assays commonly used, as well as the advantage of developing these assays in early clinical development.

11:30 Key Statistical Considerations in Interpreting USP General Chapter

Matthew Stephenson, PhD, Director of Statistics, Quantics Biostatistics

The United States Pharmacopeia (USP) General Chapter <1033> provides guidance on the validation of biological assays. We highlight key statistical considerations in the interpretation of this guidance, including setting acceptance criteria based on the probability of out-of-specification results and the use of total analytical error to quantify accuracy and precision. These reflect an evolving approach towards bioassay validation, underscoring current best practices and continual improvement.

12:00 pm PANEL DISCUSSION: Getting Down to Bioassay Brass Tacks

Moderator: Nancy Sajjadi, Independent Quality Consultant, Sajjadi Consulting

Uncertainty about how regulatory authorities determine the suitability of potency assays still persists. Successful bioassay development takes into consideration (1) current regulatory requirements and guidance, (2) the nature of the product, and (3) the statistical tools needed to evaluate decisions. This session is intended to stimulate dialogue about the challenges faced by stakeholders in the development of bioassays to support CGT products and increase clarity about current regulatory thinking.

Panelists:

Dawn Maier, PhD, Cell and Gene Therapy CMC, AD Preclinical Technical Advisor, DGMAIER Consulting
Matthew Stephenson, PhD, Director of Statistics, Quantics Biostatistics
Leslie Wagner, Consumer Safety Officer, FDA CBER
Jennifer Kirk, PhD, Lead Mathematical Statistician, FDA CBER

12:30 Luncheon Presentation *(Sponsorship Opportunity Available)* **or Enjoy Lunch on Your Own**

1:00 Session Break

IN VITRO ASSESSMENTS FOR IMMUNOGENICITY

1:30 Chairperson's Remarks

Kevin Gurney, PhD, Distinguished Scientist, Analytical Research and Development, Merck

1:35 A Mechanism-Based Dendritic Cell Migration Assay to Predict Subcutaneous Immunogenicity

Nicole Jarvi, PhD, Senior Scientist, Merck

Subcutaneous (SC) delivery can exacerbate immunogenicity of biologics where injected protein is exposed to skin-derived dendritic cells (DC) migrating into the injection site and toward lymph nodes. In a newly developed, mechanism-based dendritic cell migration assay, chemokine receptor expression on DCs and their *in vitro* migration correlated strongly with clinical immunogenicity incidence. This assay can inform decision-making of SC immunogenicity risk, with applicability to screen formulation and quality attributes.

2:05 FEATURED PRESENTATION: Immunogenicity Risk Assessment of Biotherapeutic Attributes Using *in vitro*, *in vivo*, and Clinical Data

Marisa Joubert, PhD, Scientific Director and Group Leader, Amgen

Understanding the risk of immunogenicity of Critical Quality Attributes (CQAs) is crucial to ensuring the safety and efficacy of biotherapeutic drug products. There are a variety of approaches and model systems for assessing risk that leverage *in vitro*, *in vivo*, and clinical data. This talk will share a few case studies assessing risk of immunogenicity of biotherapeutic attributes and the model systems employed.

2:35 Immunogenicity Risk Assessment and Mitigation Strategy for Biotherapeutic Lead Selection

Yinyin Li, PhD, Principal Scientist, Biochemical & Cellular Pharmacology, Genentech, Inc.

To understand, manage, and minimize the impact of immunogenicity on the clinical tolerance and activity of drug discovery, we've established a suite of in-vitro tools for immunogenicity risk assessment and mitigation during therapeutic lead identification and optimization. I will share a case study demonstrating the application of multiple tools in assessing immunogenicity risk. You will gain insights into how they can be used to drive decision-making for therapeutic lead selection.

BIOASSAY STRATEGY FOR NEW MODALITIES

3:05 Neutralization Antibody Assay for Antibody-Drug Conjugate (ADC): Killing or Non-Killing?

Weifeng Xu, PhD, Director, Bioanalytical, Merck

All approved ADCs have cell-killing NAb assays and industrial consensus is consistent with this. Multiple MOAs exist *in vivo* for ADCs; however, *in vitro* NAb assays can only monitor target-involved internalization and killing. In addition, artifacts may exist for *in vitro* cell-killing NAb assays, for example, FcgR-involved non-specific killing. We thus propose that like mAb therapeutics, cell-binding or competitive ligand-binding NAb assays could be considered for ADC.

4:05 Close of Summit

TS1A: A Guide to Statistical Methods for Bioassay*

OCTOBER 16-17, 2024

**Available in-person only*

TS1: A Guide to Statistical Methods for Bioassay

Instructor:

David Lansky, PhD, President, Precision Bioassay, Inc.

This course introduces statistical ideas supporting bioassays (illustrated with relevant examples) and reviews properties of bioassays. The course covers how practical constraints in the laboratory create the 'statistical design structure' of assays. Adapting design of experiments (DOE) to bioassay development, validation, and monitoring involves using the assay design structure. Examples (mostly from cell-based bioassays; some using robotics) illustrate strategic approaches to early development, measuring assay capability, improving a bioassay, and doing validation. Strategic assay design considerations combine with good assay analysis methods to offer good assay monitoring with graphical and quantitative tools as part of a lifecycle approach.

David Lansky, PhD, President, Precision Bioassay, Inc.

David has been practicing statistics on bioassays (and other non-clinical applications in Pharma) for 35 years. This includes Searle/Monsanto/Pharmacia (10 years) and as the owner of Precision Bioassay, Inc. (since 2002). Most of his bioassay experience involves helping teams improve and validate cell-based bioassays. His experience includes some early (late 1990's) success as part of a team that used lab automation for a series of cell-based bioassays. He has been and is still an active participant in the work to revise the USP bioassay chapters. His education includes a year of Electrical and Computer Engineering (University of Michigan), a BS in Botany (San Francisco State), an MS in Entomology (Cornell) and finally both an MS and Ph.D. in Biometry (both Cornell).

See the website for agenda & further details

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Hotel & Travel Information

Conference Hotel and Venue:

The Watergate Hotel
2650 Virginia Ave NW
Washington, D.C. 20037

Discounted Room Rate: $309 s/d

Discounted Room Rate Cut-off Date: September 16, 2024

Reservations and Additional Travel Information:
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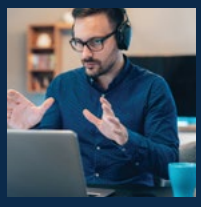
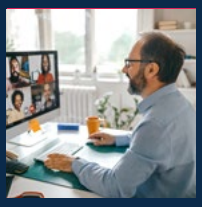
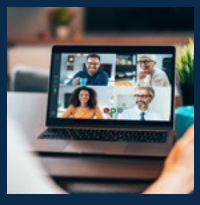


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