January 24, 2023

08:00 - 08:30

CASSS Welcome and the 11th Annual William Hancock Award

Presented by: Julia Edwards*, Genentech, a Member of the Roche Group,* CASSS President

08:30 - 08:45

WCBP 2023 Introduction

Presented by: Kenneth Miller, *BioMarin Pharmaceutical Inc.*, WCBP 2023 Symposium Co-Chair

08:45 - 10:00

Keynote Presentation

Realizing the Promise of CRISPR Therapeutics: In Vivo and Cell Therapy Applications
Laura Sepp-Lorenzino
Intellia Therapeutics, Inc., Cambridge, MA, USA

10:00 - 10:30

Networking Break

(Bloody) Marys, Mimosas & Mobile App

10:30 - 12:00

Parallel Session 1 - Implementing ICH Q12: Industry and Regulator's Experiences

Session Chairs: Kavita Aiyer, *Seagen Inc.,*
Andrew Chang, *Novo Nordisk Inc.,* Sarah Kennett, *Genentech, a Member of the Roche Group*

10:30 - 10:55
Reviewers' Perspective from the Health Canada Pilot Program on ECs and PACMPs
Hugo Hamel
Health Canada, Ottawa, Ontario, Canada

10:55 - 11:20
Win, Lose or Draw. The ICH Q12 Experience from the Industry Perspective.
Minh Luu
Genentech, South San Francisco, CA, USA

Parallel Session 2 - Cell and Gene Therapy: New Frontier and Our Best Hope to Cure

Session Chairs: J.R. Dobbins, *Eli Lilly and Company,* Roman Drews, *Arcellx, Inc.*

10:30 - 10:55
Regenerative Medicine for the 21st Century
Steven Bauer
WFIRM, Winston-Salem, NC, USA

10:55 - 11:20
Engineering AAV Capsids to Improve Delivery of Genomic Medicines to the Central Nervous System
David Ojala
Sangamo Therapeutics, Richmond, California, USA

11:20 - 11:45
Cell and Gene Therapies: FDA's Initiatives in Accelerating Product Development
Ramjay Vatsan
CBER, FDA, Silver Spring, MD, USA

12:00 - 12:30

12:30 - 13:30

Technical Seminar Presented by Thermo Fisher Scientific

12:30 - 13:30
High-Resolution Charge Profiling of Various Biopharmaceutical Modalities Using Novel Ion Exchange Phase and LC-HRAM MS
Jonathan Bones[1], Reiko Kiyonami[2]
[1]*NIBRT, Blackrock, Co. Dublin, Ireland.*
[2]*Thermo Fisher Scientific, San Jose, CA, USA*

Technical Seminar Presented by SCIEX

12:30 - 13:30
Unifying Characterization Strategies for Novel Proteins and Gene Therapy Products or Delivery Vehicles
David Colquhoun
SCIEX, Framingham, MA, USA

Technical Seminar Presented by Agilent Technologies, Inc.

12:30 - 13:30
Improved Thermal Melt Analysis Workflow with the Cary 3500 UV-VIS Spectrophotometer
Scott Melis
Agilent Technologies, Inc., Dover, DE, USA

Technical Seminar Presented by U.S. Pharmacopeia (USP)

12:30 - 13:30
USP Standards and Tools to Support Mass Spectrometry-Based Characterization of Therapeutic Proteins
Diane McCarthy, Niomi Peckham
USP, Rockville, Maryland, USA

13:30 - 14:00

14:00 - 15:15

Plenshop Session 1 - ICH M4Q(R2)

Plenshop Co-Leads:

Susan Kirshner*, CDER, FDA*

Kathy Lee, *Genentech, a Member of the Roche Group*

Ingrid Markovic, *CBER, FDA*

Henrik Kim Nielsen, *Novo Nordisk A/S*

Additional Panel Members:

Workshop Session 1 - Implementation of ICH Q12: Successes and Challenges

Workshop Co-Leads:

Lee Bink, *GlaxoSmithKline*

Andrea George, *CDER, FDA*

Alexey Khrenov, *CBER, FDA*

Frank Maggio, *Amgen Inc.*

Workshop Session 1 - Cell and Gene Therapy: Tools for Control and Product Quality, Analytical Methods, Supply Chain, Regulatory Challenges, and Raw Materials

Workshop Co-leads:

Methal Albarghouthi, *AstraZeneca*

Christine Harman, *CBER, FDA*
John Harrahy, *Sanofi*

	Rita Algorri, *Amgen Inc*. John Harrahy, *Sanofi* Ingrid Markovic, *CBER, FDA* Geoffrey Wu, *CDER, FDA* **Update on Ich M4q(r2) and Industry Perspective** Henrik Kim Nielsen *Novo Nordisk A/S, Bagsvaerd, Denmark* **FDA's Current Effort in Structured Product Quality Submission (aka PQ/CMC)** Geoffrey Wu *US FDA, Silver Spring, Maryland, USA*		
15:15 - 15:45	**Networking Break**		
15:45 - 17:15	**Plenary Session 3 - Global Submissions and Regulatory Assessments: Towards Harmonized Approaches in the Regulation of Medicines to Patients** **Session Chairs:** Marla Abodeely, *Sanofi*, Natalya Ananyeva, *CBER, FDA,* Maria Cecilia Tami, *Genentech, A Member of the Roche Group* Session Panelists: Samvel Azatyan, *World Health Organization,* Geneva, Switzerland Christopher Downey, *CDER, FDA* Veronica Jekerle, *European Medicines Agency,* Amsterdam, Netherlands Ingrid Markovic, *CBER, FDA,* Silver Spring, MD, USA Elkiane Rama*, ANVISA*, Brasília*,* Brazil Dean Smith, *Health Canada,* Ottawa, ON, Canada Eric Karikari-Boateng, *Food and Drugs Authority,* Accra, Ghana Yasuhiro Kishioka, *PMDA-Pharmaceuticals and Medical Devices Agency,* Tokyo, Japan		
17:15 - 18:30			
18:30 - 21:30	**Welcome Reception**		

January 25, 2023

08:30 - 10:00

Plenary Session 4 - Resolving Complexity: Innovative Analytical Technologies for Characterization of Complex Modalities

Session Chairs: James Carroll, *Pfizer, Inc.,* Zahra Shahrokh, *ZDev Consulting*, Marjorie Shapiro, *CDER, FDA*

08:30 - 08:55
Characterization of Full/Empty Capsids for AAV Gene Therapy
Andrea Sobjak
Janssen Research & Development, LLC, Malvern, PA, USA

08:55 - 09:20
Lot-specific Plasma IgG Hypersensitivity Reactions: Finding a Needle in a Haystack
David Boerema
CSL Behring, Kankakee, IL, USA

09:20 - 09:45
Concurrent Manufacturability Evaluation During Lead Candidate Selection Facilitates Faster CMC Timelines to Clinic for Complex Molecules
Chris Leiske
Seagen Inc., Bothell, WA, USA

10:00 - 10:30

Networking Break

10:30 - 12:00

Parallel Session 5 - Innovations in Drug Delivery Technology to Enable the Next Generation of Biologics

Session Chairs: Mark DeStefano, *Teva Pharmaceuticals*, Nomalie Jaya, *Seagen Inc.*, Shiven Kapur, *Eli Lilly and Company*

10:30 - 10:55
Drug Delivery Devices: A Market Perspective and Technology Discussion
Mathias Romacker
Kymanox, Morrisville, NC, USA

10:55 - 11:20
Innovative Drug and Device Technologies and Their Impact on Quality Systems, Control Strategies and Risk Management.
Rick Wedge[1], Henri Akouka[2]
[1]*Pfizer, Cambridge, United Kingdom.* [2]*Teva Pharmaceuticals USA, Inc., West Chester, PA, USA*

11:20 - 11:45
Regulatory Impacts and Considerations with Innovative Drug Delivery Technologies
Courtney Evans
CDRH, FDA, Silver Spring, MD, USA

Parallel Session 6 - ICH Q14 and Q2(R2) Concepts and Enhanced Approaches to the Analytical Method Lifecycle

Session Chairs: Carmilia Jiménez Ramírez, *BioMarin Pharmaceutical Inc.,* Wayne Kelley, *GlaxoSmithKline*, Isabelle Lequeux, *BioPhorum Operations Group*

10:30 - 10:55
ICH Q14 & Related Regulatory Aspects
Most Parvin
OCBQ/CBER/FDA, Silver Spring, MD, USA

10:55 - 11:20
Platform Analytical Method Lifecycle: Trends, Strategies, and Case Study Through the Lens of mRNA Vaccines
David Ripley
Pfizer, Inc., Andover, MA, USA

11:20 - 11:45
A Vision of the Opportunities and Challenges That ICH Q2 (2) and Q14 Will Bring for Analytical Procedure Development, Validation and Maintenance and This Across the Entire Analytical Lifecycle
Jean-François Dierick
GlaxoSmithKline, Brussels, Belgium

12:00 - 12:30

12:30 - 13:30

Technical Seminar Sponsored by Bio-Techne

12:30 - 13:30
Introducing MauriceFlex™: The New Maurice Featuring iCIEF Fractionation for LCMS Charge Variant Characterization
Kefei Wang[1], Jian He[2]
[1]*Bio-Techne, San Jose, CA, USA.* [2]*AbbVie Bioresearch Center, Worcester, MA, USA*

Technical Seminar Sponsored by Cygnus Technologies

12:30 - 13:30
Advanced Orthogonal Methods to Fully Characterize Process HCPs
Eric Bishop, Alla Zilberman, Jared Isaac
Cygnus Technologies, Southport, NC, USA

Technical Seminar Sponsored by Wyatt Technology

12:30 - 13:30
Light Scattering Tools for Quantifying Viral Vector Critical Quality Attributes
John Champagne
Wyatt Technology, Goleta, CA, USA

Technical Seminar Sponsored by Catalent Biologics

12:30 - 13:30
Biologics Analytical Methods: Trends and Strategies to Accelerate Biologics Development
Wai Lam Ling
Catalent Biologics, Somerset, New Jersey, USA

13:30 - 13:45

13:45 - 14:45

Roundtable Session 1

Topics:
1. Comparability for CGTP and Other Complex Modalities

Mini Case Studies Session 1

Topics:
1. Risk Versus Benefit: Acceleration of CMC for Biologics/Vaccines Products

	2. Setting Commercial Specifications Beyond Clinical Experience 3. Acceleration and CMC - Novel Approaches to Enabling Acceleration, Lessons Learned, Challenges Being Faced 4. Comparability Strategies for Protein Based Biotherapeutics 5. FDA's Request for a Control Strategy Table to Support Organization and Analysis of the Information Required to Assess Whether Manufacturing Processes Are Under Control 6. Best Practices to Design Forced Degradation Studies / Design of Stability Studies for Accelerated Product Development 7. Developing the Right Drug Product Strength – Challenges with MaPP 5019.1 and 5019.2 8. Regulator Review Preferences and Recent Review Trends: Questions and Key Issues 9. Potency Assays and Use of Structure Function Models: Replacing in-vivo with in-vitro Potency Assays at Warp Speed 10. Reference Standards: Common Practices and Challenges 11. Technical Transfer of Analytical Methods, particularly within sites and building 12. Continuous Manufacturing for Biologics 13. Using Bayesian Statistics in Stability Modeling, Leveraging Prior Knowledge 14. Table 14: Beyond the Science: Diversity, Equity and Inclusion 15. Emerging Technology: PAT, Modular Manufacturing, Real Time Release Testing 16. Host Cell Proteins: Identification and Monitoring 17. Particulates - Visible and Sub-visible: New Analytical Technologies and Requirements 18. ICH Q12- Regulatory Insights, Successful Use of PACMP, Case Studies- What Worked, Didn't		2. Just the Two of Us: Emerging Trends in Combination Products	
14:45 - 15:15	**Networking Break**			
15:15 - 16:30	**Plenshop Session 2 - ICHQ14/Q2(R2) - Concepts and Enhanced Approaches to the Analytical Method Lifecycle** Plenshop Co-Leads: Nina Cauchon, *Amgen Inc.* Christof Finkler, *F. Hoffmann-La Roche Ltd.* Amy Hsu, *CDER, FDA* Tao Pan, *CBER, FDA* **FDA/CDER Perspectives on Analytical Procedure Development and Validation** Amy Hsu *CDER, FDA, Silver Spring, Maryland, USA* **Cell and Gene Therapies: Next-Generation Therapies have Next-Generation Analytical Challenges** Mary Beth Pelletier *Biogen, Cary, NC, USA*	**Plenshop Session 2 - Modelling Tools for Stability, Molecular Design, Comparability, and Formulation** Plenshop Co-Leads: Lori McCaig, *Seagen Inc.* Ashutosh Rao, *CDER, FDA* Arne Staby, *Novo Nordisk* Joey Studts, *Boehringer Ingelheim Pharma GmbH & Co. KG*	**Plenshop Session 2 - BioPhorum Collaborations: Best Regulatory Practices for Lifecycle Management** Plenshop Co-Leads: Kavita Aiyer, *Seagen Inc.* Isabelle Lequeux, *BioPhorum* Pamela Pegman, *Pfizer, Inc.*	**Workshop Session 2 - One Site to Rule Them All vs the Multiverse of Sites – Will Distributed Drug Supply Strategies Introduce a Paradigm Shift for Biologics?** Workshop Co-Leads: Riley Myers, *CDER, FDA* Graham Tulloch, *Janssen Research & Development, LLC* Magnus Schroeder, *Just – Evotec Biologics, Inc.*
16:30 - 16:45				
16:45 - 18:15	**Plenary Session 7 - Knowledge Management and Sharing – You Don't Know What You Know** Session Chairs: Nina Cauchon, *Amgen Inc.,* Carol Krantz, *Seagen Inc.,* Joseph Kutza, *AstraZeneca* **16:45 - 17:10** **Harnessing the Invaluable Tribal Knowledge Hidden in Your Organization** Martin Lipa *Merck & Co., Inc., West Point, PA, USA. Pharmaceutical Regulatory Science Team, Dublin, Ireland* **17:10 - 17:35** **Taking Knowledge Management Into the Cloud: Using Structured Data in a Cloud-Based Ecosystem to Enable Reusability, Agility, and Interoperability**			

	Rita Algorri *Amgen, Thousand Oaks, CA, USA* **17:35 - 18:00** **FDA's KASA and PQ/CMC Initiatives: Perspectives for Biotechnology Products** Bazarragchaa Damdinsuren *CDER,FDA, Silver Spring, MD, USA*
18:15 - 19:30	**Exhibitor Reception**

January 26, 2023

09:00 - 10:30

Plenary Session 8 - The Wonders and Woes of Biological Platforms

Session Chairs: Markus Blümel, *Novartis Pharma AG*, Edwin Moore, *University of Illinois*, Timothy Schofield, *CMC Sciences, LLC*

Additional Panelists:

09:05 - 09:20
Sequence Agnosticism as the Basis of Prior Knowledge Justifications
Jack Kramarczyk
Moderna, Inc., Somerville, MA, USA

09:20 - 09:35
Regulatory Perspective on the Applicability of Platform and Prior Knowledge in Product Development
Maria Teresa Gutierrez Lugo
US-FDA, Silver Spring, MD, USA

09:35 - 09:50
Pyramids, Puddings, Fingerprints, and Frogs: Looking Back on 20 years of Platform Development
Jeffrey Baker
Jeffrey C. Baker Consultations, Washington, DC, USA

Philip Krause
Independent Consultant, Bethesda, MD, USA

10:30 - 11:00

Networking Break

11:00 - 12:00

Roundtable Session 2

Topics:
19. Comparability for CGTP and Other Complex Modalities
20. Setting Commercial Specifications Beyond Clinical Experience
21. Acceleration and CMC - Novel Approaches to Enabling Acceleration, Lesson Learned, Challenges Being Faced
22. Comparability Strategies for Protein Based Biotherapeutics
23. FDA's Request for a Control Strategy Table to Support Organization and Analysis of the Information Required to Assess Whether Manufacturing Processes Are Under Control
24. Best Practices to Design Forced Degradation Studies / Design of Stability Studies for Accelerated Product Development
25. Developing the Right Drug Product Strength – Challenges with MaPP 5019.1 and 5019.2
26. Regulator Review Preferences and Recent Review Trends: Questions and Key Issues
27. Potency Assays and Use of Structure Function Models: Replacing in-vivo with in-vitro Potency Assays at Warp Speed
28. Quality Risk Management for Cross-Contamination in Multi-Product Facilities
29. Process Technology Transfer: Opportunities and Challenges
30. Extractable and Leachable Studies: Best Practices to Support New Products and Process Changes
31. Multi-attribute Methods (MAM) and New Analytical Technologies
32. Reference Standards for Cell & Gene Therapy Products- Best Practices for Autologous Therapies
33. NMR Fingerprinting in Late-stage Development
34. RNA- Vaccines and Therapeutics
35. Microbial Challenge In-use Studies and Requirements
36. Cell and Gene Therapy Products - Manufacturing Control Strategy and CQAs

Mini Case Studies Session 2

Topics:
1. Implementation of Novel Strategies and Novel Technologies for Acceleration of IND and BLA Submissions
2. Q12 Topics and Applicability to Global Markets: Challenges for Global Expansion
3. China Case Study- Clinical Trial Requirements, CMC-specific

12:00 - 12:30

12:30 - 13:30

Technical Seminar Sponsored by Waters Corporation

12:30 - 13:30
Addressing Increasingly Complex Biotherapeutics with Advances in Analytical Workflows
Nick Pittman
Waters, Manchester, United Kingdom

Technical Seminar Sponsored by ProtaGene

12:30 - 13:30
Complex Characterization Challenges and Analytical Development Solutions for AAV Products
Chen Li, Yang Shen
ProtaGene US, Inc., Burlington, MA, USA

Technical Seminar Sponsored by Genedata

12:30 - 13:30
Accelerating Bioprocess Development and Process Analytics Through Digital Innovation
Jana Hersch
Genedata, Lexington, MA, USA

13:30 - 14:00				
14:00 - 15:15	**Workshop Session 3 - Advanced Manufacturing Technological/MAM (QC Turnaround Improvements)** Workshop Co-Leads: Kelley Burridge, *CDER, FDA* Tura Camilli, *Genentech, a Member of the Roche Group* Shawn Novick, *IABS-International Alliance for Biological Standardization*	**Workshop Session 3 - Hot Topic - Host Cell Proteins** Workshop Co-Leads: Sarah Kennett, *Genentech, a Member of the Roche Group* Nadine Ritter, *Global Biotech Experts, LLC*	**Debate (Workshop) Session 3 - Platform Technologies Anissa Cheung, Carol Krantz** Workshop Co-Leads: Anissa Cheung, *CBER, FDA* Carol Krantz, *Seagen Inc.* Session Panelists: Patrick Swann, *CSL Behring* Swapnil Bhargava, *AbCellera* Arthur Hewig, *Gilead Sciences, Inc.* Catherine Eakin, *Seagen Inc.*	**Workshop Session 3 - Potency, with a Vx-focused Discussion on Away from Animal to in-vitro** Workshop Co-Leads: Robin Levis, *CBER, FDA* Tim Schofield, *CMC Sciences, LLC* Bob Sitrin, *PATH*
15:15 - 15:45	**Networking Break**			
15:45 - 17:15	**Plenary Session 9 - Next Generation Therapeutics and Vaccines** Session Chairs: Lauren Carter, *University of Washington,* Bharat Dixit, *Adiso & ClearB Therapeutics,* Leslie Wagner, *CBER, FDA* **15:45 - 16:10** **Computational Protein Design of Nanoparticle Vaccines - Update and Overview** Lauren Carter *University of Washington, Seattle, WA, USA* **16:35 - 17:00** **Exploring Advanced Drying Techniques for Manufacturing Next Generation Drug Products** Francis Kinderman *Amgen Inc., Thousand Oaks, CA, USA*			
17:15 - 17:30	**Acknowledgements, Closing Remarks & Invitation to WCBP 2024** Presented by: Kevin King, *Pfizer, Inc.,* WCBP 2024 Symposium Co-Chair			