CMC Winter 2025

Schedule

Monday, 27 January, 2025

07:00-08:30 Promenade Foyer / Senate Room

Registration for CMC Strategy Forum North America Winter 2025

Registration is open from 07:00 to 17:00
Pre-registered attendees should go to the Foyer. If you are registering onsite, please make your way to the Senate Room.

07:30-08:30 Palm Court Ballroom

Rise and Dine: Breakfast

Breakfast will be served until 09:00.

08:30-09:00 Grand Ballroom

CASSS Welcome and CMC Strategy Forum 2025 Introductory Comments

Natalie Ciaccio, Marc Verhagen
Live Streamed
Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

Forum Co-Chairs: Natalie Ciaccio, *Vir Technology* and Marc Verhagen, *Sanofi*

The generation of technical data to support the development and manufacture of pharmaceutical products and associated regulatory submissions is fundamental to the pharmaceutical industry. The recent boom in artificial intelligence (AI) systems and large language models (LLM) has resulted in increased interest in the capability of these systems to support industry activities. By embracing technological advancement, the biopharmaceutical industry can make significant strides in reducing submission timelines and accelerating the delivery of therapeutics. This session will include discussion on current practices with examples of how structured data, digitalization, AI, and generative AI (GenAI) are being leveraged to support pharmaceutical development, manufacturing, and standardized regulatory authoring/submission with dynamic review. The focus will be on opportunities to improve efficiency or accelerate development timelines. Discussion will include considerations for emerging technologies and forward-looking approaches for analytical data modeling.

Different aspects of the use of AI or LLMs during CMC development will be discussed including:

- Use of AI in drug discovery/developability – modeling of protein structure and properties based on sequence
- Bioprocess/ cell culture optimization using predictive analysis
- Stability modeling using AI and/or machine learning
- Leveraging commercial manufacturing data/modeling to optimize process conditions
- Automated generation of regulatory documents using LLM

08:30-09:00 District Ballroom

CASSS Welcome and CMC Strategy Forum 2025 Introductory Comments

Fiona Cornel, JR Dobbins, Shawn Novick, Douglas Richardson
Live Streamed
Advances Toward Patient-Centric Quality Standards

Forum Co-Chairs: Fiona Cornel, *Health Canada,* JR Dobbins, *Eli Lilly and Company,* Doug Richardson, *Merck & Company, Inc.,* Shawn Novick, *BioPhia Consulting*

Quality standards is a term which encompasses management of quality from prior to manufacturing through patient dosing. Quality standards are applied in facility design, process design, product release, storage, and dosing. Quality standards are essential to develop and execute appropriate manufacturing process and product control strategies, that ultimately ensure that biopharmaceuticals remain safe, effective, and of consistent quality. A patient-centric quality standard (PCQS) has previously been defined as a set of patient-relevant attributes and their associated acceptance ranges to which a drug product should conform within the expected patient exposure range (see A. Mire-Sluis et al., J. Pharm. Sci. 2024, Vol 113, 837-855).

New tools and enhanced understanding of biopharmaceuticals, including prior knowledge, allow Sponsors to identify and better understand the safety and efficacy profiles for many critical quality attributes (CQAs). This understanding provides an opportunity to set requirements and ranges for manufacturing and release that are patient-centric rather than mainly reflecting manufacturing experience. Therefore, achieving patient-centricity is understanding patient-relevance, which is defined as the level of impact that a quality attribute could have on safety and efficacy within the potential exposure range.
By using a patient-centric approach to setting quality standards, batches of drug product (DP) will not be rejected due to an explainable shift in a quality attribute, if the shift is within the range determined to be safe and effective for the patient. Additionally, this can increase the flexibility of sponsors to explore newer or novel technologies during product life-cycle management.

This forum will explore the tools available to understand and set patient-centric quality standards including control strategies which ensure safety, efficacy, and product availability for the patient. Advancements, challenges and case studies in the development and application of patient-centric quality standards will be presented. Regulatory considerations and how to apply them in a fashion which is consistent with regulatory guidance to ensure a robust, safe and efficacious patient experience will also be explored.

09:00-10:40 Grand Ballroom

Workshop I - Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

Natalie Ciaccio, Cortney Lawrence, Laura Pack, Marc Verhagen
Live Streamed
Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

Session Speakers:

The Challenge and Criticality of Data Contextualization to Support Effective AI/ML/Modeling in Bioprocess Development – an Industrial Case Study
Christian Airiau, Sanofi

Development of Realistic and Safe AI/ML Applications for Biotherapeutic Characterization and Process Development
Jeremy Shaver, *Pfizer, Inc.*

A "One-Click" Submission with AI and Digital Authoring
Kabir Ahluwalia, *Amgen Inc.*

Practical and Regulatory Considerations for Machine Learning Models Applied to Process Development and Control
Ben Stevens*, GlaxoSmithKline*

09:00-10:40 District Ballroom

Workshop I - Approaches to Developing an Enhanced Understanding of Product Quality Expectations

Cristiana Campa, Filippos Kesisoglou
Live Streamed
Advances Toward Patient-Centric Quality Standards

The session will explore approaches towards developing enhanced product understanding that supports the establishment patient centric quality standards and control strategies.

Speakers will present recent advances on nonclinical tools that inform criticality of quality attributes (CQAs), such as use of biotransformation data for biotherapeutics and in vivo/ in vitro models for vaccines. In addition, the application of a structured CQA assessment will be discussed, to provide insights on risk assessment strategies to ensure appropriate knowledge of product safety and efficacy, and consequent establishment of effective control strategies. The session will conclude with sharing the regulatory perspective on these approaches.

Session Speakers:

What is Meant by Patient-Centric Quality Standards?
Phil Krause, *Independent Consultant*

Patient-Centric Control Strategy Development: Principles, Tool, and mAb Examples
Claudia Gributs, *Eli Lilly and Company*

Leveraging Biotransformation Data to Refine Bioprocesses and Derisk PQAs
Maribel Beaumont, *Merck & Company, Inc.*

Defining CQAs Impacting Vaccine Efficacy: The Role of in Vitro and in Vivo Models
Barbara Capecchi, *GlaxoSmithKline*

Enhanced Approaches to Setting Specifications – A Regulatory Perspective
Jayda Siggers, *Health Canada*

10:40-11:10 Palm Court Ballroom

Coffee Connection: Networking Break

Enjoy some coffee, a snack, and network with your peers!

11:10-12:25 Grand Ballroom

Workshop I Panel Discussion - Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

Natalie Ciaccio, Cortney Lawrence, Laura Pack, Marc Verhagen
Live Streamed
Digital Transformation: AI and Data Analytics to Accelerate Development Timelines
Additional Panelist: Paula Russell, *Health Canada*

11:10-12:25 District Ballroom

Workshop I Panel Discussion - Approaches to Developing an Enhanced Understanding of Product Quality Expectations

Cristiana Campa, Filippos Kesisoglou
Live Streamed
Advances Toward Patient-Centric Quality Standards
Additional Panelist: Douglas Roepke, *Eli Lilly and Company*

12:25-14:00 Palm Court Ballroom

Meet and Eat: Hosted Lunch

Join us for a nutritious networking lunch in the Palm Court Ballroom!

14:00-15:40 District Ballroom

Workshop II - Case Studies for Applying Patient Centric Quality Standards

Markus Blümel, Cristiana Campa
Live Streamed
Advances Toward Patient-Centric Quality Standards

This session presents cases studies and practical experiences from industry on implementing the advanced concepts of patient-centric quality standards. This includes challenges observed and examples from protein therapeutics as well as cell & gene therapies. The perspective and experience from regulators will be shared. The presentations will be followed by a plenary discussion to improve mutual understanding and establish good practices to ensure a robust, safe and efficacious patient experience.

Session Speakers:

A High Threshold of Aggregate Numbers Is Needed to Induce Immunogenic Response in Vitro, in Vivo, and in the Clinic
Kimya Nourbakhsh, *Amgen Inc.*

Strategies for Defining Specifications in Autologous Cell Therapy Products
Daisy Nie, *Novartis Pharmaceuticals Corporation*

Case Studies for Patient-Centric Specification Setting for a Therapeutic Protein and IQ Readout on Endotoxin Acceptance Criteria
Katrina Kearns, *Pfizer, Inc.*

Allowable Excess Volume and Gross Content Requirements and Challenges For Injectable Drug Products in Vials
Ehab Moussa, *AbbVie Inc.*

Enhanced Approaches Support Robust Harmonized Vaccine Potency Specifications
Dean Smith, *Health Canada*

14:00-15:40 Grand Ballroom

Workshop II - Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

Andrew Lennard, Laura Pack, Paula Russell
Live Streamed
Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

Session Speakers:

Empowering 5.0 BioSolutions Through Realtime PAT and Computational Controls
Edita Botonjic-Sehic, *ReciBioPharm AB*

Predictive Stability Modeling of mRNA Vaccines
Gang Wang, *Moderna, Inc.*

AI-Accelerated Stability for Biologics
Seyma Bayrak, *Amgen Inc.*

Regulatory Expectations for Data Models to Support a Marketing Application
Jayda Siggers, *Health Canada*

15:40-16:10 Palm Court Ballroom

Coffee Connection: Networking Break

Enjoy some coffee, a snack, and network with your peers!

16:10-17:25 Grand Ballroom

Workshop II Panel Discussion - Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

Andrew Lennard, Laura Pack, Paula Russell
Live Streamed
Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

16:10-17:25 District Ballroom

Workshop II Panel Discussion - Case Studies for Applying Patient Centric Quality Standards

Markus Blümel
Live Streamed
Advances Toward Patient-Centric Quality Standards
Additional Panelist:

Fiona Cornel, *Health Canada*

17:25-17:40 District Ballroom

Closing Remarks: Invitation to CMC Strategy Forum Summer 2025

Live Streamed
Advances Toward Patient-Centric Quality Standards

17:25-17:40 Grand Ballroom

Closing Remarks: Invitation to CMC Strategy Forum Summer 2025

Natalie Ciaccio, Marc Verhagen
Live Streamed
Digital Transformation: AI and Data Analytics to Accelerate Development Timelines

17:45-19:15 Palm Court Ballroom

Networking Reception

Join us for a Networking Reception in the Palm Court Ballroom!