

# CMC Strategy Forum North America: Summer 2023

## Schedule

Monday, 17 July, 2023

07:30-08:30 Washingtonian Foyer

Registration

*Registration will be open until 17:00 pm*

07:30-08:30 Salon C

Breakfast

*Breakfast will be available until 9:00 AM*

08:30-08:45 Salons D- G

CASSS Welcome and Introductory Comments

Presented by: Shawn Novick, *IABS-International Alliance for Biological Standardization*

08:45-09:00 Salons D- G

CMC Strategy Forum Welcome and Introductory Comments

Presented by: Mike Abernathy, *Amgen Inc.*

09:00-10:30 Salons D- G

Workshop I: Digital Regulatory Filings and Assessment Enabled by Cloud-based Interoperability and Data Exchange

Co-Chairs: Mike Abernathy, Shawn Novick

As the pharmaceutical industry edges into the digital age of PHARMA 4.0, significant changes are coming for Chemistry, Manufacturing, and Controls (CMC) principles and practices, knowledge management, and data exchange or interoperability. Advancements in technological innovation have exponentially increased over the past several decades and continue to rapidly evolve as many industries pursue digital maturity. In comparison, the biopharmaceutical industry has been slower to adopt new technologies due to implementation barriers and complexities conferred by the global regulatory framework. However, patients cannot wait indefinitely for the industry to modernize regulatory processes and harmonize product quality standards. To meet patient needs for acceleration and manage changing regulatory expectations, biopharmaceutical companies and regulators must utilize up-to-date, structured, and standardized information via a cloud-based ecosystem to accelerate the product approval process. The strategic implementation of a cloud platform that ingests and exchanges structured, standardized, digital filings can transform the life sciences industry, leading to increased productivity through efficient interoperability, innovation, and improved speed of therapies to patients.

Session Speakers:

**The Genesis of M4Q(R2): A Regulatory Perspective**

Ingrid Markovic, *CBER, FDA*

**ISO/IDMP Initiative: Maturing Structure and Standardization with the Healthcare Ecosystem**

Brooke Casselberry, *NNIT, Inc.*

**The Future of Real-time Algorithmic Exchange and Processing of CMC Data Using FHIR**

Craig Anderson, *Pfizer, Inc.*

10:30-11:00 Washingtonian Foyer & Salon C

Networking Break

11:00-12:20 Salons D- G

Workshop Session I: Panel Discussion

Additional Panelist(s):

Rita Algorri, *Amgen Inc.*

Lawrence Yu, *CDER, FDA*

12:20-13:20 Washingtonian Foyer & Salon C

Networking Lunch

13:20-14:55 Salons D- G

Workshop Session II: Look Forward to Harmonization

Co-Chairs: Colleen Godshall, Kathy Lee

Day 1, session 2, "Look Forward to Harmonization" will cover the FDA's efforts to implement KASA as a tool towards knowledge management within the Agency. Day 1, session 2 will also describe the regulatory reliance pathway, which is considered the 21st century best regulatory practice as it will bring greater efficiency to the regulatory process by eliminating duplicative work, strengthen regulatory systems, and optimize resource utilization with a focus on value-added activities without sacrificing product quality. Finally, day 1 will wrap up with a presentation which will put ongoing initiatives and programs into perspective, offering both industry and regulators advance knowledge management and harmonization tools to meet the needs of patients.

Session Speakers:

**FDA Perspective for Modernization of Regulatory Assessment and Submission including KASA and PQ/CMC**

Bazarragchaa Damdinsuren, *CDER, FDA*

**Reliance - Towards a Regulatory Standard**

Cecilia Tami, *Genentech, a Member of the Roche Group*

**The Future of Regulatory Assessment and Submission: Impact of Digitalization**

Lawrence Yu, *CDER, FDA*

14:55-15:25 Washingtonian Foyer & Salon C

Networking Break

15:25-16:45 Salons D- G

Workshop Session II: Panel Discussion

Additional Panelist(s):

Sheetal Gaiki, *Janssen Research & Development, LLC*

Gang Xue, *Janssen Research & Development, LLC*

16:45-18:00 Washingtonian Foyer & Salon C

Networking Reception

Tuesday, 18 July, 2023

08:00-09:00 Washingtonian Foyer

Registration

*Registration will be open until 17:00*

08:00-09:00 Washingtonian Foyer & Salon C

Breakfast

*Breakfast will be available until 9:30 AM*

09:00-10:30 Salons D- G

Workshop Session III: Efforts to Harmonize on the Definition of Quality: Based on Patient-Centricity

Co-Chairs: Andrew Chang, Timothy Schofield

Key to the achievement of a globally harmonized control strategy is agreement on principles and practices which lead to the CMC content of the CTD for Market Authorization Application. This begins with a common view of the basis of product quality. Specification is a part of the overall control strategy to assure product safety and efficacy. "A specification is defined as a list of tests, references to analytical procedures, and appropriate acceptance criteria which are numerical limits, ranges, or other criteria for the tests described." [ICH Q6B]. Harmonization has been hampered, however, by the notion that acceptance criteria should be calculated from test results on some sampling of manufactured lots. This practice has been identified as one of the major causes of disparity across regions and has impeded practices related to quality by design (QbD).

An alternative has come to be called patient-centric specifications (PCS). This focuses the ICH definition of the specification on the patient rather than on manufacturing, resulting in an opportunity to engage in patient-centric and risk-based development and lifecycle management. This also aligns biopharmaceutical, vaccines, and C&GT businesses with other industries, which use a customer-focused definition of specifications.

This session will include talks on the principles and practices associated with PCS, featuring justifications for acceptance ranges focused on risk-based assessment of the impact to patients. The relationship between PCS and stability, process, formulation, and analytical development will be explored, as well as opportunities for strategic designs of post-approval change management protocols. The use of PCS for quality control will be contrasted with manufacturing control which uses control limits to help manage production consistency.

Attendees will be expected to engage with the speakers in discussion about patient-centric specifications, and the pathway to globally harmonized technical content in the CTD.

Session Speakers:

**A Framework for Ensuring Quality and Patient-Centric Specifications**

Tim Schofield, *CMC Sciences, LLC*

**What Was Gained and Lost with the Harmonization of Specifications with COVID-19 Vaccines**

Jason Fernandes, *Health Canada*

**Patient Focused Specifications**

Susan Kirshner, *CDER, FDA*

10:30-11:00 Washingtonian Foyer & Salon C

Networking Break

11:00-12:20 Salons D- G

Workshop Session III: Panel Discussion

Additional Panelist:

Barbara Rellahan, *Amgen Inc.*

12:20-13:20 Washingtonian Foyer & Salon C

Networking Lunch

13:20-14:55 Salons D- G

Workshop Session IV: Statistical Modeling Approaches and Implementation of Platform Prior Knowledge Provides an Opportunity for Accelerated Drug Development

Co-Chairs: Lori McCaig, Crystal Pannucci

On Day 1 we considered opportunities to improve harmonization of dossier compilation, submission and review through efficiency gains. Day 2 focuses on opportunities for patient-centric approaches to Chemistry Manufacturing and Controls, which includes accelerating product development with the goal of getting new medicines to patients faster.

The Day 2 afternoon session develops further the morning's patient-centric considerations for the use of prior knowledge (from product early development and from non-product-specific 'analogous molecules', also referred to as 'like-molecules') as a valuable tool to accelerate product development that benefits from platform technologies.

The use of platform-based therapeutic development (product type, manufacturing process, formulation, container closure etc.) has led to considerable understanding in the behavior of different molecules that are derived from the same platform. As more medicines are taken through development, identifying information that can be distilled into prior knowledge provides an opportunity to enhance the development process by prioritizing molecule-specific outcomes that have a meaningful impact on product quality. Product stability is one such activity that has drawn interest in leveraging prior knowledge to improve statistical modeling for product stability when stability data are limited through accelerated development programs.

Proper implementation of prior knowledge, though, poses multiple challenges. Does prior knowledge need to be derived only from a defined platform? As processes evolve, where is the line between enhancements within the same platform and a different platform altogether? What techniques facilitate improved understanding of true product stability, but recognize unique molecule-specific stability behavior?

This session will include talks that explore the importance of stability modeling to accelerate the CMC timeline and hopes for the ICH Stability guideline revision to further harmonize stability data expectations and shelf-life setting. It will also provide, practical considerations in using platform prior knowledge, and how Bayesian and other methodologies can help combine prior knowledge with molecule-specific data.

Attendees will be expected to engage with the speakers in discussion about how to utilize statistical models and best ways to implement platform prior knowledge to accelerate product development and bring safe, quality medicines to patients in need, sooner.

Session Speakers:

**Predictive Stability Methodologies: The Why, When and What**

Barbara Rellahan, *Amgen Inc.*

**Bayesian Statistics: Using Prior Knowledge to Enhance Understanding of Product-Specific Stability**

Adam Rauk, *Eli Lilly and Company*

**Modeling for Product-specific Stability: A Regulatory Perspective**

Paula Russell, *Health Canada*

14:55-15:10 Washingtonian Foyer & Salon C

Mini-Break

15:10-16:15 Salons D- G

Workshop Session IV: Panel Discussion

Co-Chairs: Lori McCaig, Crystal Pannucci

Additional Panelist:

Ashutosh Rao, *CDER, FDA*

16:15-16:35 Salons D- G

Feature Presentation: Making Harmonization a Reality

The pandemic has taught us how critical vast patient access necessitates the need for the adoption of reliance and collaborations between health authorities and industry. Over day 1 and day 2, we have talked about the advancements of technological innovation and an aim for globally harmonized control strategy and prior knowledge for CMC submissions.

The final talk will provide a holistic perspective on the acceptance of a harmonized CMC submission by health authorities around the world through the use of Reliance.

Featured Speaker:

Kathy Lee

*Genentech, a Member of the Roche Group*

16:35-16:50 Salons D- G

Discussion with Feature Presentation Speaker Kathy Lee

Co-Chairs: Crystal Pannucci

16:50-17:00 Salons D- G

Closing Remarks and Invitation to CMC Strategy Forum North America 2024

Presented by: Andrew Chang