CMC Strategy Forum Japan 2025

Schedule

Monday, 8 December, 2025

07:30-08:30 Foyer

Coffee Service

Presentation type: IP - In Person

Start your day right with our morning Coffee Service! Coffee will be served until 09:00.

Enjoy freshly brewed coffee and tea while connecting with fellow attendees in a relaxed, welcoming atmosphere. It's the perfect opportunity to ease into the day and spark meaningful conversations!

07:30-08:30 Foyer

Registration for CMC Strategy Forum Japan

Presentation type: IP - In Person

Registration will be open until 16:30.

08:30-08:45 Ballroom North AB

CASSS Welcome and Introductory Comments

Wassim Nashabeh
Presentation type: IP - In Person

08:45-09:00 Ballroom North AB

CMC Strategy Forum Japan 2025 Welcome and Introductory Comments

Yoshiaki Maruyama
Presentation type: IP - In Person

09:00-10:20 Ballroom North AB

Session I - Recent Trends in the Regulation of Biopharmaceutical Products

Alexey Khrenov, Ryosuke Kuribayashi
Presentation type: IP - In Person

In this session, regulators from various health authorities will provide the regulatory updates and future perspective regarding biopharmaceutical products, including regenerative medicine products.

Building on the experience gained from expedited development and approvals in response to COVID-19, as well as the ICMRA joint pilot review, there is a growing demand for more flexible approaches to drug development and regulatory review Furthermore, the advancement of drug discovery for wide variety of modalities including ATMPs, as well as increasing use of advanced manufacturing technologies have made the establishment of a new review approaches an increasingly important challenge.

Against this background, ICH M4Q (R2) aims to improve submission and assessment efficiency, providing benefit to both industry and regulatory agencies, and driving accelerated access to pharmaceuticals for patients.

The presentations will include information that will contribute to, and be further explored, in a panel discussion covering several topics, including:

Key Questions:
- Need for changes and structural reforms in the application system to enable more flexible and expedited reviews
- Progress in mutual reliance and joint reviews/inspections for biopharmaceutical products regulators, including lessons learned from past experiences, and future perspectives and initiatives (e.g., ICMRA/PQKMS, ASEAN, ACCESS, Project OBIS)
- The evolution of regulatory frameworks to fit new modality products including ATMPs
- Regulatory updates relevant to biopharmaceuticals products/cell and gene therapy products including latest guidelines (e.g. Platform Technology Designation Program for Drug Development)
- Modernization of marketing authorization application dossiers in the future: For example, perspectives on harmonization initiatives such as the ongoing M4Q revision and "Structured Product Quality Submissions" being carried out at ICH.
- Progress, issues, and future perspective for ICH Q12 implementation: e.g., Established Conditions, PACMP, PLCM.

Session Speakers:
Regulatory Updates and a Perspective on Biologics in Japan
Ryosuke Kuribayashi, *Pharmaceuticals and Medical Devices Agency (PMDA)*

ICH Q12 Implementation in Japan, and Current Situation of ICH M4Q(R2)
Keisuke Tanaka, *Pharmaceuticals and Medical Devices Agency (PMDA)*

Regulatory Update From European Medicines Agency (Virtual Presentation)
Nino Mihokovic, *European Medicines Agency (EMA)*

PQS Effectiveness, ICH Q9(R1) & Using the CMC Regulatory Tools of ICH Q12: a European Regulator's Perspective
Kevin O'Donnell, *Health Products Regulatory Authority (HPRA) Ireland*

10:20-10:50 Foyer

Morning Networking Break

Presentation type: IP - In Person

10:50-12:05 Ballroom North AB

Session I - Panel Discussion: Questions & Answers

Alexey Khrenov, Ryosuke Kuribayashi
Presentation type: IP - In Person

Additional Panelists:

Marcel Hoefnagel, *Medicines Evaluation Board (MEB)*

Jayda Siggers, *Health Canada*

12:05-13:25 Ballroom South CD

Buffet Lunch

Presentation type: IP - In Person

13:25-14:50 Ballroom North AB

Session II - Advancements in AAV technology: Quality Considerations, Analytics and Manufacturing Strategies

Srinivasan Kellathur, Yasuhiro Kishioka
Presentation type: IP - In Person

Adeno-associated virus (AAV) is gaining attention as a promising tool for gene therapy and vaccines, with many clinical and commercial products emerging recently. Evaluating the quality of AAV products is essential for understanding their characteristics, developing manufacturing processes, assessing storage and stability, and ensuring consistency throughout their lifecycle. Due to their complexity, AAV vector products require various analytical methods, but recent advancements have improved these technologies significantly.
This session will explore the latest analytical techniques, their applications in process development, approaches for quality evaluation at different development stages, strategies for consistency, and key regulatory considerations.
Key Questions:
1. HEK cells remain the primary production system for AAV manufacturing, but challenges such as characterization, productivity enhancement, viral risk management, and quality control persist.
a. What innovative strategies can enhance the productivity and quality of AAV manufacturing using HEK cells?
b. How can we standardize and advance HEK cell characterization methods to meet regulatory and quality requirements across the lifecycle?

2. There are ongoing challenges in balancing impurity management through advanced analytical techniques and improving recovery rates via purification methods.
a. How can analytical advancements contribute to effective impurity management without compromising the recovery yield during purification?
b. What are the key challenges in balancing high recovery rates and stringent purity requirements, and how can new technologies help overcome them?

3. Empty or partially filled AAV capsids present a significant challenge in AAV-based gene therapy, leading to undesirable immune responses and diminish therapeutic efficacy. Empty-to-full capsid ratio is a critical quality attribute (CQA) in AAV vector production. While traditional approaches offer some control in managing this ratio, they are often inefficient. This necessitates extensive downstream purification processes to separate empty and partial capsids from full ones, adding complexity and cost to manufacturing.
a. What are the key innovations in purification techniques and process optimization, specifically those aimed at reducing empty capsids?
b. What are the regulatory considerations such as thresholds for empty capsid ratio and safety assessments?

4. Fed-batch vs continuous manufacturing (CM) in AAV production. Fed-batch production presents several challenges, including high variability, extended processing times, and limitations in scalability. In contrast, continuous manufacturing offers significant advantages such as increased efficiency and productivity, reduced processing times, enhanced process consistency, and improved product quality.
a. Share your experiences on CM regarding scalability, improved throughput and product consistency both in upstream and downstream processing.
b. The viral vector production is a major driver of manufacturing cost. Could CM reduce overall production cost?

5. Introducing high-throughput sequencing, computational models coupled with artificial intelligence and machine learning tools have significant potential in capsid engineering, analytics and purification strategies.
a. Any developments on the application of such novel technologies in AAV manufacturing?

6. Regulator's expectations regarding comparability strategies for gene therapy products

Session Speakers:

rAAV as a Heterogeneous Particle Ensemble: Current Understanding of Its Quality Attributes
Susumu Uchiyama, *The University of Osaka*

Characterising Viral Vectors for Gene Therapy Using Mass Spectrometry on Different Levels
Jonathan Bones, *NIBRT*

Challenges of Manufacturing of AAV for Gene Therapy Products, Focusing on Upstream and Downstream Processes
Takashi Sakurai, *Astellas Pharma, Inc.*

Quality Considerations in the Development of AAV Vector Products
Kenichiro Maeda, *Pharmaceuticals and Medical Devices Agency (PMDA)*

14:50-15:20 Foyer

Afternoon Networking Break

Presentation type: IP - In Person

15:20-16:40 Ballroom North AB

Session II - Panel Discussion: Questions and Answers

Srinivasan Kellathur, Yasuhiro Kishioka
Presentation type: IP - In Person

16:40-18:10 Ballroom South CD

Networking Reception

Presentation type: IP - In Person

Tuesday, 9 December, 2025

07:30-08:45 Foyer

Coffee Service

Presentation type: IP - In Person

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07:30-08:45 Foyer

Registration for CMC Strategy Forum Japan

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Registration will be open until 16:30.

08:45-10:10 Ballroom North AB

Session III - Advancing Stability Testing: Modernisation and Expansion of the ICH Q1 Guideline with Science- and Risk-Based Approaches

Akiko Ishii-Watabe, Andrew Lennard
Presentation type: IP - In Person

The draft guideline ICH Q1 "Stability Testing of Drug Substances and Drug Products" reached Step 2b of the ICH process on April 11, 2025, marking the start of the public consultation period as Step 3. This revised draft adheres to the fundamental principles of the current ICH Q1/Q5C while introducing important new elements that are expected to have a broad impact on the practical aspects of pharmaceutical development, manufacturing, and quality control across the product life cycle. Key updates include the incorporation of risk-based and scientifically grounded approaches to advanced stability testing designs, as well as considerations for evaluating in-use stability. In addition, the draft also suggests the possibility of establishing retest period for drug substance and shelf-life extrapolation for biologics, similar to the approach used for chemical drugs, which represents a noteworthy element for future implementation. The revisions to ICH Q1 and Q5C have been previously addressed as session topics in past CMC Strategy Forum Japan, with a particular focus on aspects related to biologics, such as the potential use of modeling in stability prediction. This session will revisit the revised draft guideline overview, and deepen discussions on critical updates and key considerations such as proposals for flexible stability testing approaches. This discussion will also incorporate perspective from pharmaceutical industry with a focus on challenges to be addressed to facilitate future implementation, including the development of training materials.

Key Questions:

Regulatory Harmonization and Future Prospects

- Risk - and science-based flexible approaches are expected to become available. However, since this is a relatively new initiative, it is still unclear whether regulatory authorities in each region share a common understanding of what kinds of data sets would support such approaches and enable for streamlining, or whether challenges to harmonization still persist.

- What benefits are expected from the new concepts introduced in the draft guideline for industry, authorities, and patients, respectively?

- Regarding the potential for the use of stability modeling in biopharmaceuticals, what are the future prospects of this approach?

Specific Points to Consider for Stability Testing

- Regarding in-use stability newly added in the draft guideline, are there any points that remain unclear or open to discussion in the current draft?

- Are there any additional consideration required for the test methods for stability testing compared to that for DS and DP.

- Although the draft guideline does not specify particular points for ADC, are there aspects that should be considered specifically for the stability testing for ADC?

- As for ADCs and certain type of bispecific antibodies, the antibody component may be treated as an intermediate. If antibody is treated as an intermediate, what points should be considered in stability testing?

Statistical Methods and Extrapolation
- Are there any points to consider or recommendations regarding the use of extrapolation in establishing shelf life for biopharmaceuticals?

- With the newly introduced statistical methods in the draft guideline (such as mixed-effects models and advanced stability modeling), what changes are anticipated in shelf-life setting for biopharmaceuticals, and are there any case studies?

Session Speakers:

Overview of the ICH-Q1 Guideline Revision Draft and Prospects for Stability Assessment of Biological Products from the Regulatory Perspective
Takashi Kameda, *Pharmaceuticals and Medical Devices Agency (PMDA)*

ICH Q1s/5C Revision – Opportunities in Science and Risk-Based Testing Approaches
Boris Zimmermann, *Genentech, a Member of the Roche Group*

“Tradition Is a Guide Not a Jailer”: Standard Versus Alternative Approaches in the ICH Q1 Revision
Andrew Lennard, *Amgen Limited (UK)*

Challenges and Approaches Related to Extrapolation and Shelf Life/re-Test Period Setting in Biological Products
Hiroko Shibata, *National Institute of Health Sciences (NIHS)*

10:10-10:40 Foyer

Morning Networking Break

Presentation type: IP - In Person

10:40-11:55 Ballroom North AB

Session III - Panel Discussion: Questions & Answers

Akiko Ishii-Watabe, Andrew Lennard
Presentation type: IP - In Person
Additional Panelist:

Akiko Ishii, *National Institute of Health Sciences (NIST)*

11:55-13:10 Ballroom South CD

Buffet Lunch

Presentation type: IP - In Person

13:10-14:35 Ballroom North AB

Session IV - Innovative Approaches in Biopharmaceutical Manufacturing: Advanced Manufacturing, Dx, and AI/ML

Alexey Khrenov, Yosuke Watanabe
Presentation type: IP - In Person

This session will explore innovative approaches in biopharmaceutical manufacturing and quality control, focusing on Advanced Manufacturing, Digital Transformation (Dx), and Artificial Intelligence/Machine Learning (AI/ML). The presentations will share the regulatory perspective on innovation and present the latest case studies covering automation and optimization of manufacturing processes, enhancement of quality control, real-time monitoring, predictive maintenance, and AI applications in the development phase.

The session will also be used to discuss challenges and solutions from the perspectives of regulatory compliance and data integrity, sharing practical insights that contribute to industry-wide transformation.

Key Questions for Discussion:

- What are the regulatory challenges for the use of AI/Dx in manufacturing, as well as other advanced approaches (e.g. continuous manufacturing, process modeling, distributed manufacturing)?

- What expectations or concerns exist regarding the use of Advanced Manufacturing and AI in communication between Health Authority and the Industry

- What existing initiatives (e.g.. industry organization, international collaboration) can foster wider acceptance of Advanced Manufacturing/DX/AI in biopharmaceutical sector?

- How is consistency with GMP regulations and ICH guidelines ensured when applying AI to quality system (e.g. anomaly detection, predictive maintenance)?

- What changes are expected in controls strategies of manufacturing, and in organizational structures with the introduction of Dx and AI?

- How to ensure data governance and security in the utilization of AI?

- How to enable development of AI skills in the manufacturing and quality departments? What kind of support is necessary for on-site operators and technicians to efficiently use AI and automation technologies?

- What areas are AI and modeling currently utilized in, or could be utilized in, for the development and commercial manufacturing process of biopharmaceuticals, including continuous manufacturing process?

- Does progress in AI and modeling help drive greater utilization of continuous manufacturing?

- What are the key considerations for utilizing AI and modeling in process control for continuous manufacturing?

- What is expected to be the most significant innovation in biopharmaceutical manufacturing over the next 5 years?

Session Speakers:

Industry Perspective on Implementation of AI and Machine Learning in Manufacturing - Emerging Regulatory Landscape
Gert Thurau, *F. Hoffmann-La Roche Ltd. (Virtual Speaker)*

Continuous Manufacturing and Portable On-Demand (POD) for Biologics: Development Challenges and Regulatory Considerations
Kenichiro Furuki, *Merck Sharp & Dohme*

Regulatory Perspectives on Model-Informed Decision-Making to Support Biologic Drug Development and Marketing Applications
Jayda Siggers, *Health Canada*

EMA Quality Innovation Group (QIG): Two Years Experience in Regulatory Support of Innovation in Pharmaceutical Manufacturing
Marcel Hoefnagel, *Medicines Evaluation Board (MEB)*

14:35-15:05 Foyer

Afternoon Networking Break

Presentation type: IP - In Person

15:05-16:20 Ballroom North AB

Session IV - Panel Discussion

Alexey Khrenov, Yosuke Watanabe
Presentation type: IP - In Person

Additional Panelists:

Kevin O'Donnell, Health Products Regulatory Authority (HPRA) Ireland

Akira Sakurai, *Pharmaceuticals and Medical Devices Agency (PMDA)*

Kosuke Takenaka, *Takeda Pharmaceutical Company Limited*

Yosuke Watanabe, *Chugai Pharmaceutical Co., Ltd.*

16:20-16:35 Ballroom North AB

Closing Remarks and Invitation to CMC Strategy Forum Japan 2026

Jamie Moore
Presentation type: IP - In Person