SCIENTIFIC Conference

e-Book

International Alliance for Biological Standardization

IABS Workshop on Global Harmonization of Specification: Implementing A Patient-Centric, Enhanced Control Strategy

June 23-25, 2025

TOKYO, JAPAN

www.iabs.org

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About the Conference

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A key component for a biologicals control strategy to ensure safety and efficacy is the product specification. Current regulatory guidelines include general principles regarding establishing specifications, with a focus on analytical technologies used for batch release and stability testing but provide limited detail about science and the use of risk-based approaches to define a product specification. This creates regional differences in regulatory requirements and their interpretations, which increases the complexity of product supply chain, and decreases patient access in global distribution.

In the fourth IABS workshop focused on global harmonization of specifications, we expand further the discussion on what has been called patient-centric product specifications and is now being referred to as 'enhanced' approaches to setting specifications. This approach further aligns specification setting with development knowledge and risk-based decision making, considering the role of the release specification in the overall quality control strategy. The meeting will discuss potential specific considerations for different product modalities (e.g., vaccines, biotherapeutics, and cell/gene therapies), lifecycle management, and the global regulatory landscape on specification requirements. Case studies and tools used to support enhanced, risk-based specifications will be shared and discussed. In addition, a key question relevant to all product modalities will be, in the absence of safety and efficacy issues with a marketed product, is there scientific justification for tightening a specification based on increased manufacturing consistency?

This workshop compliments other activities and organizations working towards the goal of global harmonization and improving access of biologicals.
Participants in this IABS workshop will gain understanding of the challenges and opportunities to global harmonization of specifications through the discussion of tools and solid examples where the enhanced approach to setting specifications – i.e. specifications which help ensure safety and efficacy of the drug product by using risk management, process knowledge, and analytical understanding – were successful in preparing and receiving approval for a biologic.

Scientific and Organizing Committee

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Scientific Committee

Shawn Novick - Co-Chair, IABS, USA
Kelley Burridge - CDER-FDA, USA
Cristiana Campa - GSK, Italy
Andrew Chang - Novo Nordisk, USA
Gerald Gellermann - Novartis, Switzerland
Melody Gossage - Lilly, USA
Akiko Ishii - National Institute of Health Sciences, Japan
Michael Jordan - MSD, Ireland
John Kim - Bill & Melinda Gates Medical Research Institute, USA
Andrew Lennard - Amgen, UK
Yoji Sato - Co-Chair, National Institute of Health Sciences, Japan
Robin Levis - CBER-FDA, USA
Mourad Mellal - Catalent, Belgium
Bart van Montfort - Johnson & Johnson, Netherlands
Akira Sakurai - PMDA, Japan
Dean Smith - Health Canada, Canada
Cecilia Tami - Genentech, USA
Emily Xianghong - CDER-FDA, USA
Satoshi Yasuda - National Institute of Health Sciences, Japan

Organizing Committee

Shawn Novick - Co-Chair, IABS, USA
Yoji Sato - Co-Chair, National Institute of Health Sciences, Japan
Dean Smith - Health Canada, Canada
Madinina Cox - Events Manager - IABS/MC'Com, France

Upcoming IABS Conferences and Workshops

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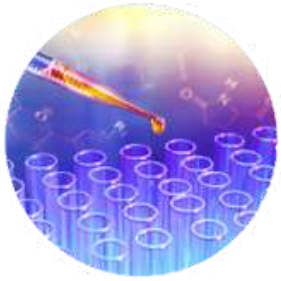
11th Annual Statistics Workshop
Big Tent Statistics – Conveying The Importance Of Statistical Contributions

Rockville, MD, USA
October 22-24, 2025



AFSA – IABS Conference about Animal testing replacement for vaccines: A One Health View: global outlook and future strategy

Bangkok, Thailand
December 2-4, 2025

Advances in Analytical Technologies for Biopharmaceutical Products

Rockville, MD, USA
March, 2026

Scientific Program

Monday, June 23, 2025

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8:00 - 8:30 Registration and Welcome Coffee

8:30 - 8:45 **Welcome address**
IABS & Chairs

KEYNOTE SESSION

8:45 - 9:25 **Strategy for Retinal Cell Therapy**
Masayo Takahashi, Vision Care, Japan

SESSION 1 - Common/general Principles of using the Patient Centric or Enhanced approach to setting specifications for a product, including modality considerations

Moderators: Dean Smith, Health Canada & Cristiana Campa, GSK, Italy

9:25 - 10:05 **Summary of outcomes and key messages from previous GHS meeting**
Phil KRAUSE, USA

10:05 - 10:30 *Coffee & Tea Break*

Scientific Program

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10:30-11:00

ICH Q6 review status
Akiko ISHII-WATABE, ICH Q6 EWG, NIHS, Japan

11:00-11:30

Industry perspective on ICH Q6 review, with focus on Japan/Asia region opportunities
Takahiro YAMAGUCHI, ICH EWG member for JPMA, Japan (Virtual)

11:30-12:15

Panel Discussion
Phil KRAUSE, USA
Akiko ISHII-WATABE, ICH Q6 EWG, NIHS, Japan
Andrew CHANG, Novo Nordisk, USA
Horacio PAPPA, US Pharmacopeia, USA

12:15-1:45

Lunch Break

SESSION 2 - Case studies: Can the enhanced approach be successful? How do we justify ranges and what is the feedback encountered?

Moderators: Robin Levis, CBER-FDA, USA, Gerald Gellermann, Novartis, Switzerland & Jayda Siggers, Health Canada, Canada

In this session different case studies will be presented to illustrate ways to an enhanced product understanding. The product understanding builds the basis for the definition of product limits that ensure safety and efficacy. The case studies will also focus on illustrating how the generated knowledge on the potentially critical and non-critical attributes can be used for process consistency monitoring or as product-specifications in an overall control strategy to ensure pharmaceutical quality.

Scientific Program

Monday, June 23, 2025

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1:45-2:10
Science and Risk-based approaches to Specifications: Application to rapid development of Antibody-drug conjugates
Keiko FUNATO, GSK, Japan

2:10-2:35
Vaccines are Biologicals with Unique Specificities
Bénédicte MOUTERDE & Patrice RIOU, Sanofi, France

2:35-3:00
Addressing Uncertainties in Complex Biologics: Enhanced vs. Conventional Approaches
Gerald GELLERMANN, Novartis, Switzerland

3:00-3:25
A Clinical Impact of Attributes (CIA) Approach for developing clinically relevant specifications for biologics
Marisa JOUBERT, AMGEN, USA

3:25-3:50
Challenges in Product Specifications with Asian Regulatory Authorities: A Case Study
Vanessa AUQUIER, UCB, Belgium

3:50-4:15
Coffee Break

4:15-5:20
Panel Discussion
Keiko FUNATO, GSK, Japan
Bénédicte MOUTERDE, Sanofi, France
Gerald GELLERMANN, Novartis, Switzerland
Marisa JOUBERT, AMGEN, USA
Jayda SIGGERS, Health Canada, Canada
Vanessa AUQUIER, UCB, Belgium

5:20-5:30
Wrap-up

Scientific Program

Tuesday, June 24, 2025

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8:00-8:15 Registration and Welcome Coffee

8:15-8:20 **Welcome address**

SESSION 3 - Opportunities and Challenges for Global Harmonization of Product Specifications with Multiple Pharmacopoeia in a Global Landscape.

Moderators: Dean Smith, Health Canada & Akiko Ishii-Watabe, National Institute of Health Sciences, Japan

8:20-8:45 **Global landscape of specifications: a European Pharmacopoeia perspective**
Emmanuelle CHARTON, EDQM, France

8:45-9:10 **Setting Specifications: Impact of Regulatory Harmonization**
Patrice RIOU & Bénédicte MOUTERDE, Sanofi, Global

9:10 - 10:30 **Panel Discussion**
Emmanuelle CHARTON, EDQM, France
Anuradha GUPTA, India Pharmacopeia, India
Patrice RIOU, Sanofi, Global
Bénédicte MOUTERDE, Sanofi, Global
Minkyung KIM, US Pharmacopeia, South Korea (Virtual)
Hiroko SHIBATA, NIHS, Japanese Pharmacopoeia, Japan
Sirichair KRABESRI, Thai Pharmacopeia, Thailand (Virtual) - TBC
Sasiwimon PATASEMA, Thai Pharmacopeia, Thailand (Virtual) - TBC
Jayoung KIM, Korean Pharmacopeia (Virtual)

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10:30-11:00 *Coffee break*

SESSION 4 - Considerations for Cell and Gene Therapy Products and their Impact on Lifecycle Management

Moderator: Andrew Chang, Novo Nordisk, USA & Bart van Monfort, Johnson & Johnson, Netherlands

11:00-11:30 **Strategies for Defining Specifications in Autologous Cell Therapy Products**
Daisy NIE, Novartis, USA (Virtual)

11:30-12:00 **Specification consideration for CGT products and role of analytics: Challenges and Opportunities**
Kaushik SARKAR, Novo Nordisk A/S, Denmark

12:00-1:15 *Lunch Break*

1:15-1:45 **QbD-based CGT products manufacturing and lifecycle management**
Shin KAWAMATA, Cyto-Facto, Japan

1:45-2:30 **Panel Discussion**
Shin KAWAMATA, Cyto-Facto, Japan
Kaushik SARKAR, Novo Nordisk A/S, Denmark
Daisy NIE, Novartis, USA (Virtual)
Yoji SATO, NIHS, Japan

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2:30 - 3:00 *Coffee break*

SESSION 5 - Break Out (Part 1)

3:00 - 5:00 When is it appropriate to tighten or broaden specification ranges? What experiences have we encountered? Is this different depending upon the modality/risk/benefit?

- **Vaccines**

Moderator: Dean Smith, Health Canada, Canada

- **Biotherapeutics**

Moderator: Shawn Novick, IABS, USA

- **Cell & Gene Therapy**

Moderator: Bart Van Montfort, Johnson & Johnson, Netherlands

- **Online Break Out**

Moderator: Cristiana Campa, GSK, Italy

5:00 - 5:15 Wrap-Up

Scientific Program

Wednesday, June 25, 2025

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8:00 - 8:30	Registration and Welcome Coffee
8:30 - 8:45	**Welcome address**

SESSION 6 - Break Out (Part 2)

8:45 - 10:00	**Break Out Session Summary** **Panel Discussion**
10:00 - 10:30	*Coffee break*
10:30 - 11:45	**SESSION 7 – Panel discussion** **Regulators and Industry: What is the path forward? What actions are occurring globally to allow for 'one product-one specification/quality standard'?**
11:45 - 12:15	**Meeting review & Summary**
12:15	**Closing remarks**

Biosketch

Vanessa Auquier, PhD

Head of Specifications, Clinical Release and Stability

Analytical Development Sciences / UCB

Address: Chemin du Foriest, 1420 Braine-l'Alleud. Belgium

Tel: +32 496 82 23 32

E-mail: vanessa.auquier@ucb.com

Vanessa Auquier is managing the "Specifications, Clinical Release and Stability" team within the Analytical Development Sciences Department at UCB. She graduated in Bio-Engineering from the University of Brussels and earned a PhD in Cell Biology, focusing on membrane protein structure and function.

Vanessa joined UCB in 2007, where she became part of the Analytical Development team specializing in method development and validation for biomolecules. Leveraging her analytical expertise, she advanced to the role of Analytical Product Lead for both clinical and commercial biomolecules, managing the entire analytical package (method lifecycle, stability, and specifications).

Since 2017, Vanessa has been leading a dedicated team responsible for the strategies for clinical and initial commercial specifications for biomolecules, as well as clinical release and stability strategies. In 2024, the scope of her team expanded to include chemical molecules.

Abstract

Vanessa Auquier

Challenges in Product Specifications with Asian Regulatory Authorities: A Case Study

In the pharmaceutical industry, navigating regulatory requirements across different regions can be particularly challenging. This presentation focuses through a case study on the difficulties encountered with certain Asian authorities, specifically in South Korea and China, regarding the specifications of our products.
Traditionally, Size Exclusion High-Performance Liquid Chromatography (SE-HPLC) has been used for the testing of the high molecular weight species (HMWS), monomer (purity) and low molecular weight species (LMWS) for monoclonal antibodies. However, our data indicates that Non-Reducing Capillary Gel Electrophoresis (NR-CGE) provides a more accurate representation of purity for our products. SE-HPLC fails to achieve baseline resolution between monomer and LMWS due to the close mass of heavy chain-light chain (HHL) species to the monomer. This leads to underestimation of LMWS and overestimation of monomer.

The literature confirms also that NR-CGE is becoming the method of choice over SE-HPLC across the industry. NR-CGE offers optimal separation between monomer and LMWS, ensuring accurate purity assessment and maintaining product quality. Despite presenting historical data and external evidence supporting the transition from SE-HPLC to NR-CGE, the reviewers remain unconvinced. They argue that impurities and monomer should be tested in a single method, achieving a total of 100% when combined. Their expectation persists regardless of product specificities and method performances.
In conclusion, although 16 countries had already accepted the strategy to test and report the monomer and LMWS by NR-CGE, we had to implement additional specifications for the monomer by SE-HPLC to avoid delaying patient access in these Asian countries. By sharing this case study, we hope to highlight the importance for the authorities to revise their standard approaches and expectations. Harmonizing specifications across regions will not only improve product quality but also expedite patient access to essential medications.

Biosketch

Kelley Burridge, PhD

Director,
CMC RA department

Akasaka Intercity AIR, 1-8-1, Akasaka,
Minato-ku, Tokyo, 107-0052, Japan

Regulatory Affairs/ GlaxoSmithKline K.K.

E-mail: kelley.burridge@fda.hhs.gov

Dr. Kelley Burridge currently serves as a product quality team leader in the Office of Pharmaceutical Quality (OPQ) in the Center for Drug Evaluation and Research (CDER) at the US FDA. She leads a team of reviewers in the assessment of chemistry, manufacturing, and controls (CMC) information for pre- and post-market human therapeutic biologic drugs. Dr. Burridge is a member of the OPQ Emerging Technology Team and ICH Q6 FDA working group.

Previous FDA positions include a chemistry reviewer in the Office of Life-cycle Drug Products (2014-2019) and lead reviewer of plastic and reconstructive surgery devices in the Center for Devices and Radiological Health (CDRH, 2010-2014). As a chemistry reviewer she assessed the quality of liquid-based drug products including topical semisolids, injectables, and peptides. Device review experience includes tissue adhesives, tissue markers, wound dressings, hemostatic agents, sutures, surgical meshes, and negative pressure wound therapies. Prior to joining the FDA, she obtained postdoctoral training experience and worked as an industrial process engineer. Dr. Burridge received a B.S. in Chemical Engineering from Cornell University and a Ph.D. in Biomedical Engineering from Boston University with special training in Biomolecular Pharmacology.

Biosketch

Cristiana Campa, PhD

Vaccines Technical R&D Advisor
GSK via Fiorentina 1, Siena, Italy

Tel: +393475737147
E-mail: cristiana.x.campa@gsk.com

Cristiana Campa, PhD, is currently a Technical R&D Advisor at GSK Vaccines, with more than 20 years' experience in Chemistry, Manufacturing and Control (CMC) in biologics research and development. In her current role, she is very active in CMC advocacy, with contributions to cross- company discussion on innovative technologies, specifications setting and accelerated development strategies, fostering dialogue with Regulatory Agencies. She is active in several trade associations and is an elected member of the Parenteral Drug Association Board of Director since January 2023. She is also a member of the ICH Expert Working Group dedicated to ICH Q6 Guidelines (Specifications) revision, as EFPIA Lead.

After her PhD and Post-Doc in Chemistry, she worked at Bracco Imaging SpA (2002-2006), first as a senior researcher and then as head of Trieste research laboratory. She joined Novartis Vaccines in 2006, Technical R&D; first as analytical senior manager and then as Head of Analytical Development, Italy. Since 2012, Cristiana has worked on Quality by Design (QbD) principles implementation across different company sites in Europe and US. After acquisition of Novartis Vaccines by GSK in 2015, she has been the Head of QbD Integration and, until June 2018, the Head of Science and Development Practices in Global Technical R&D, covering Quality by Design, Knowledge Management and Development roadmaps.

Biosketch

Dr. Andrew Chang

VP, Quality and Regulatory Compliance, Regulatory Policy and Intelligence, Global Regulatory Affairs
Plainsboro Township, NJ 08536, États-Unis
Novo Nordisk
800 Scudders Mill Road

E-mail: awcg@novonordisk.com

Dr. Andrew Chang is a multifaceted quality and CMC leader with 29 years well-rounded medical product regulatory and industry experiences. He is a board director for CASSS-Sharing Science Solutions and PDA, respectively. Biopharmaceutical Advisory Board (BioAB). At his current capacity as a Vice President, Quality and Regulatory Compliance, Regulatory Policy and Intelligence, Global Regulatory Affairs, Novo Nordisk, he provides strategic leadership on Regulatory and Quality related Policy, External Affairs, strategic advice and solutions to quality and regulatory related challenges.

Since 2013, Andrew has represented Novo Nordisk at several work groups in industry trade organizations, e.g., PhRMA and BIO to advocate patient and industry's interests by developing position papers and participating liaison meetings with the regulatory authorities. He is the topic lead from BIO for ICH Q6(R1) EWG on Specifications.

Prior to industry, Andrew had served more than 11 years in US FDA most recently as an Associate Director for Policy and Regulation, Acting Deputy Director, Lab Chief and Senior Regulatory Scientist in the Division of Hematology, CBER. He was responsible for management of marketing

Biosketch

Dr. Emmanuelle Charton

Head of Division
67000 Strasbourg, France
EDQM
Address: 7 Allée Kastner

Tel: (+33)388413640
E-mail: emmanuelle.charton@edqm.eu

Emmanuelle CHARTON holds a PhD in Biochemistry from the Institut National Agronomique de Paris-Grignon. Since 2006 she is Head of Division B in the European Pharmacopoeia department at the European Directorate for the Quality of Medicines and HealthCare (EDQM). The Scientific Secretariat for the elaboration of European Pharmacopoeia texts related to biologicals and microbiology chapters fall under the responsibility of her division. Her work experience includes QA/QC in a facility for the production of parenteral products and preparation to GMP inspections in a global pharmaceutical company, research and development in biochemistry in a global company selling food and chemicals. She has over 30 years' experience at the EDQM.

Abstract

Emmanuelle Charton

Title: Global landscape of specifications: a European Pharmacopoeia perspective

The European Pharmacopoeia (Ph. Eur.) provides legally binding public standards for the 39 member states of the Council of Europe and the European Union. While these standards are mandatory, their implementation—particularly in the field of biologicals—allows for a degree of flexibility. This presentation will illustrate how such flexibility can be applied through specific examples.

The Ph. Eur. also plays an active role in global harmonization efforts through its participation in two key international platforms: the Pharmacopoeial Discussion Group (PDG) and the International Meeting of World Pharmacopoeias (IMWP). These collaborations reflect a shared commitment among pharmacopoeias worldwide to move toward convergence and, ultimately, harmonization of public standards.

The presentation will conclude with a case study on pyrogenicity testing, highlighting the Ph. Eur.'s initiative to phase out the rabbit pyrogen test and reduce reliance on animal-derived reagents in bacterial endotoxin testing. These efforts aim not only to advance sate of the art and ethical testing practices in Europe but also to influence global standards.

Biosketch

Keiko Funato

Director,
CMC RA department

Akasaka Intercity AIR, 1-8-1, Akasaka,
Minato-ku, Tokyo, 107-0052, Japan

Regulatory Affairs/ GlaxoSmithKline K.K.

Tel: + 81 7045753646
E-mail: keiko.2.funato@gsk.com

Since 2011, working at GlaxoSmithKline K.K. and Leading & Managing of new drug applications and lifecycle management for all GSK products in the CMC section, including monoclonal antibodies (including ADCs), vaccines, and small molecules. Active member of several professional committees such as the PhRMA Science & Regulatory CMC Subcommittee in Japan (PhRMA CMCSC), the Japan Agency for Medical Research and Development Bio Subcommittee (AMED), and the JPMA Biopharmaceutical Committee (JPMA Bio).

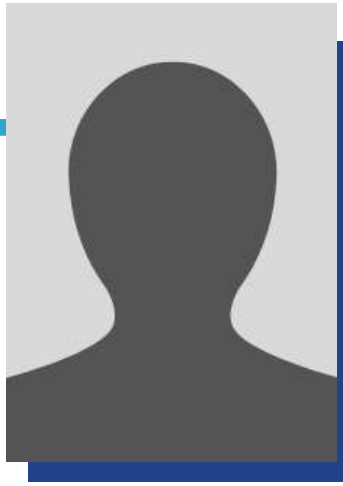
Abstract

Keiko Funato

Title Science and Risk-based approaches to Specification control: Application to rapid development of Antibody-drug conjugates

The talk will use the example of rapid development of ADC treatments for critical medical need to consider how specifications can be developed based on scientific principles and prior knowledge, and based on consideration of the overall control strategy. Consideration will be given requirements for small molecule linker/payload and mAb intermediates for ADCs. The talk will reflect on principles developed and published collaboratively across industry, and will include relevant GSK case studies.

Biosketch



Gerald Gellermann

Scientific Officer
Novartis Technical Research and Development (TRD) Biologics
Fabrikstrasse 2, 4056 Basel, Suisse

E-mail: gerald.gellermann@novartis.com

Gerald currently works as Scientific Officer at Novartis Technical Research and Development (TRD) Biologics. He is member of the Novartis ICHQ12 implementation team and leads the TRD biologics QbD and Control Strategy initiative. Prior to joining Novartis, he gained professional experience in CMC and analytical development during his time at Roche and in neuroscience research and diagnostic division at Abbot/AbbVie. Gerald holds a Master's degree in Biology from the University of Constance and a PhD in Molecular Biology from the University of Jena (both Germany). During his professional career Gerald always worked on QbD principles and cross-site implementation. At Novartis he has been head of QbD integration and leader for Science and Development Strategies, Knowledge Management and Development Roadmaps as well as for definition of Analytical Requirements and Specifications. Especially during his time as Analytical Project Leader and his current role as Scientific Officer he gained in-depth experience for definition of appropriate specifications to control process performance as well as impact of Critical Quality Attributes on patient safety and product efficacy.

Abstract

Gerald Gellermann

Title : Uncertainties in Complex Biologics: Enhanced vs. Conventional Approaches

Traditional methods primarily emphasize maintaining process consistency. In contrast, advanced approaches leverage the understanding of structural-function relationships. This presentation will cover:

- The distinction between limits for analytical procedures and Critical Quality Attributes.
- Utilizing product characterization data to establish meaningful limits that ensure safety and efficacy

Biosketch

Melody Gossage

Executive Director
Indianapolis, USA
Eli Lilly and Company

E-mail: gossage_melody@lilly.com

Melody Gossage is an Executive Director at Eli Lilly in Bioproduct Research and Development. With a extensive tenure of 20 years at the company, Melody has primarily focused on developing an integrated control strategy for large molecules, ensuring the highest standards of quality and efficacy. Throughout her career, Melody has amassed extensive experience with monoclonal antibodies, contributing significantly to their analytical development and optimization at Lilly. In recent years, she has expanded her expertise to include cell and gene therapies, reflecting her adaptability and commitment to staying at the forefront of bioproduct innovation.

Biosketch

Anuradha Gupta, PhD

Senior Scientific Officer
Indian Pharmacopoeia Commission
Ministry of Health and Family Welfare,
Govt. Of India
Sector 23, Raj Nagar, Ghaziabad 201002
India

Tel: +91-120-2783401, 2800500
E-mail: anuradha.gupta86@gov.in

Dr. Anuradha Gupta is currently working as Senior Scientific Officer at Indian Pharmacopoeia Commission, Ministry of Health and Family Welfare, Govt. Of India. Dr. Gupta is currently involved in the Development, monitoring and harmonization of monographs, general chapters and general requirements in the categories of Biotechnology derived therapeutic products (rDNA-based therapeutics), Vaccines and immunosera for human and veterinary use, Blood and blood related products, and Allergens for Indian Pharmacopoeia (IP) in consultation with stakeholders. She is also working towards implementation of '3 R' principles and activities related to 'Alternatives to animal methods.' She is also responsible for Monitoring and maintenance of QMS related activity for compliance to ISO/IEC 17025 and ISO/IEC 17043:2023 and further Testing/analysis related activities. She has13 years of experience, having interdisciplinary knowledge of pharmaceutical sciences and biological sciences. She has skilled research experience on the development of novel therapeutic strategies- host-directed therapies, synthetic lethal therapy, bimodal therapy, immunotherapies, combined chemo-immunotherapies, and nano vaccines to combat infectious diseases and cancer. In addition, she has published 35+ research papers, patent, received national and international grant and various fellowships.

Biosketch

Dr. Akiko Ishii-Watabe

Director of Division of Biological Chemistry and Biologicals

NIHS-National Institute of Health Sciences
3-25-26 Tonomachi, Kawasaki-ku,

Kawasaki City, Kanagawa, 210-9501, JAPAN

Akiko is a director of Division of Biological Chemistry and Biologicals in the National Institute of Health Sciences, Kanagawa, Japan. She received her B.S., M.S. and Ph.D. degrees in Pharmaceutical Sciences from Kyoto University. She joined NIHS in 1996. She has experiences with characterizations and bioanalysis of biopharmaceuticals. She is engaged in regulatory science research for biopharmaceuticals including therapeutic proteins and extracellular vesicle products, and a chair of the Expert committee for biologics of the Japanese Pharmacopoeia. Currently, she is a topic leader of MHLW/PMDA for ICH Q6(R1) (Specifications) Expert Working Group.

Abstract

Dr. Akiko Ishii-Watabe

Title: ICH Q6 (R1) review status

ICH Q6B guideline "Specifications: Test Procedures and Acceptance Criteria for Biotechnological/Biological Products" was harmonized in 1999 and has been a global standard for setting specifications of biologicals. However, many new modalities are being developed, and the diversity of modality is expanding. In addition, after Q8 guideline, science and risk-based approaches have been incorporated in each ICH Quality guideline. Since revision of Q6B together with Q6A for chemical entities was considered to be useful for more efficient product development and enable access to high-quality, safe and effective products to patient world-wide, the revision of Q6A/B was decided. The Expert Working Group of Q6(R1) was organized in 2024 at ICH Fukuoka meeting. Currently, the draft document of Q6(R1) is under preparation according to its concept paper. In my presentation, an overview of the topic, and current situation of discussion in Q6(R1) EWG are introduced. Then enhanced approach for setting specifications will be presented and discussed.

Biosketch

Michael Jordan, PhD

Snr. Director (HQ) Regulatory CMC

Merck Sharp & Dohme Research GmbH (MSD)

E-mail: michael.jordan@merck.com

Dr Michael Jordan was awarded his PhD in Pharmaceutical Chemistry from the University of Dublin, Trinity College. Michael currently holds a role as Snr Director Reg CMC at MSD and Adjunct Professor at University College Dublin's School of Biomolecular and Biomedical Sciences. With a strong technical background in conjugate vaccines having worked with Wyeth, Pfizer and now MSD in their Pneumonia franchises Michael transitioned to CMC in 2014 and has submitted IND, Clinical Protocols and Manufacturing applications globally. Michael also spent time as an independent consultant supporting pharmaceutical companies in submission and audit activities together with a role in sustainable manufacturing for CEPI.

Biosketch

Marisa Joubert, Ph.D.

Executive Director
91320, Thousand Oaks, California, USA
Amgen
Address: One Amgen Center Drive

Tel: +805-447-3130
E-mail: mjoubert@amgen.com

Marisa Joubert is an Executive Director and Group Leader of the Pre-pivotal Biologics Attributes Sciences team in the department of Process Development at Amgen Inc (Thousand Oaks, CA). She oversees teams that support the development of Pre-pivotal assets including Molecule Assessment, FIH, Attribute Characterization, and Attribute Impact. She has been at Amgen since 2008 as a pharmaceutical scientist in drug product development. Prior to joining Amgen, she was a Senior Researcher at the Council for Scientific and Industrial Research in Pretoria, South Africa, where she evaluated novel therapeutic agents for treating HIV-1. She received her PhD in 2006 from the University of California, Los Angeles, in Biochemistry and Molecular Biology.

Biosketch

Shin Kawamata, MD, PhD

CEO
650-0047 Kobe, Japan
Cyto-Facto Inc.
Address: Shimin Byoin mae Bldg. #301, 2-1-11 Minatojima-minamimachi

Tel: +81-78-306-0681
E-mail: kawamata.shin@cytofacto.com

Shin studied physics at Kyoto University and medicine at Kobe University (MD) and received his Ph.D. in pathology from Kyoto University (1998). During his postdoctoral period (1998-2001), he developed mouse models of human leukemia at Systemix Co., Palo Alto, USA, and Stanford University. He became Chief Scientist at the Foundation for Biomedical Research and Innovation (FBRI, 2002-2014) and Director of the Center for Cell Therapy (2015-2023 March). In April 2023, he became CEO of Cyto-Facto Inc. as a spin-off from FBRI and Professor of Science Technology and Innovation of Kobe Univ. He is secretary of ISCT Asia (2000-present). Chair of ISCT iPSC Subcommittee. His main fields are hematology, stem cell biology and cell process.

Abstract

Shin Kawamata

Title: QbD-based Cell and Gene Therapy products manufacturing and lifecycle management

Background and Challenge

For cell and gene therapy (CGT) products, testing the cells as starting materials or analyzing the raw materials is not feasible as biological products. Therefore, the quality of the product can be determined by comprehensive tests called "Verification" just before release to ensure that the product conforms to QC criteria. Since quality of materials and processes by manual is always valuable, it is difficult to define the "permissible" variation. “Verification” is a type of QC that leaves things to chance and does not question the validity of the criteria for release nor contribute to continuous improvement of raw material control, process development or quality of the final product.

Proposed approach

To overcome the stagnant situation in CGT manufacturing, we propose a quality by design (QbD)-based manufacturing approach. In this approach, the product developers must first have a clear image of the CGT product with efficacy substantiated by a robust efficacy assay. Then, they must set up SOPs to manufacture the product properly. Next, establish process parameters (PPs) and acceptable values alongside the PPs to confirm that the product is manufactured "as designed" by verifying that the values measured for each PP are within an "acceptable range" called the design space (DS). These PPs and DS consist of the product's CQAs for manufacturing. Finally, the validity of the predefined PPs and DS is verified by a robust efficacy assay of the product.

Conclusion

This allows us to visualize the CQAs of the product and support the continuous development of the CQA description at each stage of the product's life cycle. This approach accepts for variance in the quality of raw materials, changes in manufacturing scale, and technical development, as long as the manufactured product retains the same efficacy. We believe that a QbD-based approach to manufacturing CGT products would facilitate the seamless development of CGT products.

IABS

Biosketch

JaYoung Kim, Ph.D.

Deputy Director
28159 Cheongju, Republic of Korea
Ministry of Food and Drug Safety
Address: 187 Osongsaengmyeong 2-ro, Osong-eup

Tel: +82-43-719-4604
Fax: +82-43-719-4600
E-mail: jayoungkim@korea.kr

JaYoung Kim has been working at the Ministry of Food and Drug Safety(MFDS) since 2004, accumulating over 20 years of experience. She currently serves as the Deputy Director of Drug Research Division, Pharmaceutical and Medical Device Research Department. Her primary responsibilities including the revising Korean Pharmacopoeia, drug standardization, and responding to public inquiries. Previously, She spent more than 10 years in the review division, where she was responsible for evaluating the safety and efficacy data of new drugs and generics submitted by pharmaceutical company. She has also conducted research on risk assessment for drugs, including quasi-drugs and cosmetics.

In addition to her professional experience, Dr. Kim received her Ph.D. in Hygiene pharmacy and Toxicology from Ewha Womans University in 2011. She is also a licensed pharmacist in Korea.

Biosketch

John Kim

Analytical Development Lead
02139 Cambridge, (MA), United States
Bill & Melinda Gates Medical Research Institute
1 Kendall Sq Bldg 600

E-mail: john.kim@gatesmri.org

John Kim is a dedicated professional at Gates MRI (Medical Research Institute), contributing to the organization's mission of accelerating the development of life-saving interventions for global health challenges. With a focus on innovative research and collaborative approaches, John plays a pivotal role in advancing projects aimed at addressing infectious diseases, particularly in underserved populations. His expertise, combined with a commitment to impactful healthcare solutions, underscores his contributions to Gates MRI's goals of improving lives worldwide.

Biosketch

Phil Krause

Independent Consultant

Bethesda, MD 20817 USA
E-mail: phil@drkrause.com

With over 30 years of experience at the Food and Drug Administration, Phil has a unique combination of scientific, regulatory, clinical, and public health experience. He is trained as a physician with board certification in internal medicine and infectious diseases and a researcher with over 100 publications on topics spanning clinical evaluation of vaccines, viral pathogenesis and immunology, and biological product development. He has served as a key advisor to the World Health Organization, providing advice on vaccine development and evaluation, including as Chair of the WHO's Research and Development Blueprint COVID-19 Vaccine Expert Group. He is currently an independent consultant, providing strategic and regulatory advice related to biological product development.

He has made significant contributions to previous IABS meetings, most recently as a co-author of an article entitled "A vision for patient-centric specifications for biologicals", which summarized recent thinking presented by others and himself at various meetings in the past few years.

Abstract

Phil Krause

Title: Summary of outcomes and key messages from previous GHS meetings: Meeting the challenge of clinically relevant specifications

Using a scientific basis for setting and maintaining specifications can provide major benefits to patients, regulators, and developers. A clinically relevant (or patient-centric) definition of quality is used to assure that specifications are connected to product quality. Various data sources can inform the definition of quality, including clinical studies, preclinical studies, and prior information. Once quality has been defined, it is possible to select the broadest range of specifications (accounting for assay variability and stability) that are consistent with quality. Alternatively, a similar approach may be used to assure that any desired specification range is consistent with the definition of quality. Critically, specifications should not be used to monitor post-licensure process variability, which is more reliably and appropriately monitored by other elements of the integrated control strategy. Indeed, manufacturing process knowledge may contribute more to an understanding of product quality than individual test results, which are subject to assay variability. By maintaining clinical relevance as the driver for specification development, it is possible to improve international harmonization and outcomes for all stakeholders.

Biosketch

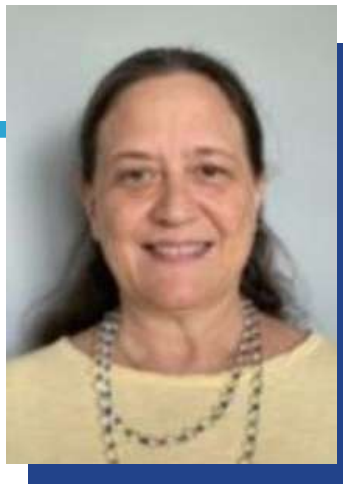
Andrew Lennard

Regulatory Affairs CMC
Uxbridge UB8 1DH, Royaume-Uni
Amgen
Business Park, 4 Sanderson Rd, Denham

E-mail: alennard@amgen.com

Andrew Lennard is in the Global Regulatory Affairs CMC team at Amgen and is based in the UK. Within CMC Regulatory Affairs, he is part of the External Engagement and Advocacy team with responsibilities in advancing innovations to accelerate CMC in product development. Andrew has over 18 years' experience in CMC Regulatory Affairs, with a special interest in control strategy and using prior knowledge, in which he has participated at the EMA workshops on Prior Knowledge, and on CMC acceleration in Breakthrough/PRIME. He is also an active member of EFPIA, leading several initiatives relating to CMC acceleration, including 'Stability' for which Andrew is the EFPIA topic lead on the Expert Working Group for the ICH Q1revision of the stability guidelines, and as part of the ICH Q6 Specifications revision EFPIA Support team. Andrew presented on case studies for approaches to a Patient-centric Specification at the 3rd IABS workshop (2023). Prior to Regulatory Affairs, Andrew was a Principal Scientist in drug discovery for large pharma and small biotech start-up companies and holds a PhD from the University of Cambridge (UK).

Biosketch



Robin Levis, Ph.D

Deputy Director, Division of Viral Products
OVRR/CBER/US FDA
10903 New Hampshire Avenue
Building 52 – Room 1208
Silver Spring, Maryland 20993, U.S.A.

Tel: +01-240-402-9665/+01-301-412-3410
E-mail: robin.levis@fda.hhs.gov

Dr. Robin Levis has worked at the US Food and Drug Administration since 1995. She is currently the Deputy Director of the Division of Viral Products in the Office of Vaccines Research and Review at CBER/FDA; a position she has held since 2006. Prior to this position, she served as the Regulatory Coordinator for the Division of Viral Products (2002-2006) and served as a Senior Staff Fellow in the Laboratory of Vector Borne Viral Diseases (1995-2002). Her initial research work at the FDA related to flavivirus replication and the role of the NS1 protein. She then transitioned to be the lead CMC reviewer for licensed rabies virus vaccine products and rabies vaccine and related products under development. Her work with rabies virus vaccines was related to the development of an alternative, in vitro potency assay as an alternative to the currently licensed NIH potency test.

In addition to her work in the Office of Vaccines at CBER, she serves as the CBER representative to ICCVAM, as an observer to EDQM Group 15 for vaccines, and serves on several vaccine working groups for the Coalition for Pandemic Preparedness Innovations. Her role on these International working groups is to provide regulatory support to CMC development and product quality.

Biosketch

Mourad Mellal

Director of Statistics
1120 Bruxelles, Belgique
Catalent Pharma Solutions
Font St Landry 10

Tel: +32 496 12 80 59
E-mail: mourad.mellal@catalent.com

Mourad Mellal is Director of Statistics at Catalent from end of March 2021. He received a Bachelor of Science degree in mathematics and computer science in 2008, a Master of Science degree in statistics in 2009 from Joseph Fourier University, Grenoble, France, and a Research Technological Diploma in applied and industrial mathematics in 2010 under a collaborative effort between Joseph Fourier University and Atomic Energy Commission.

He worked at the French research center of cancers, Institute Curie, and He joined GSK vaccines in 2011 where He had increasing responsibilities. He started as a statistical consultant within industrialization department supporting statistics for processes and analytical methods development, validation and transfer. He moved to Quality Control department in 2013 managing statistics for quality operations and He became a leader of Quality Control and Global Industrial Operations Statistical team in 2016.

Biosketch

Bénédicte Mouterde, Pharm D

CMC Regulatory Science Expert
69280 Marcy l'Etoile, France
R&D/ Sanofi Vaccine
Address: 1541 avenue Marcel Mérieux

Tel: +33 6 75 42 87 79
E-mail: benedicte.mouterde@sanofi.com

Bénédicte Mouterde has been CMC Regulatory Science Expert at Sanofi Vaccines in Analytical Research and Development for 18 years. At Sanofi, she has a transversal role in CMC regulatory intelligence, knowledge management, and advocacy for analytics. She is also involved in the definition of the analytical strategies for new vaccines. She is part of working groups at EFPIA/Vaccines Europe, CEPI and IQ Consortium. Prior to that she has a 13-year experience in regulatory affairs working on several types of vaccines and in clinical development. Bénédicte is a Doctor in Pharmacy from the University of Lyon, France.

Abstract

Benedicte Mouterde and Patrice Riou

Title: Vaccines are Biologicals with Unique Specificities

The enhanced approach/Quality by Design (QbD) represents the current preferred strategy for CMC process development and control strategy implementation for biologics, including vaccines.

However, vaccines possess unique characteristics that distinguish them from other biological products, particularly biotherapeutics and monoclonal antibodies. These specificities include their mechanism of action (immunogenicity as the basis for efficacy rather than a safety risk), route of administration, and dosing regimens.

Current regulatory frameworks and pharmacopoeial standards sometimes fail to account for these vaccine-specific attributes, applying requirements more suitable for other biologics and/or less applicable to the enhanced approach based on risk analysis and prior knowledge.

In this presentation, we will discuss concrete examples of CQAs that may be managed differently for vaccines as compared to other biologics, such as process-related impurities, subvisible particles, uniformity of dosage/content, and potency.

The implementation of QbD principles for vaccines requires recognition that while all specification tests address Critical Quality Attributes (CQAs), not all CQAs necessitate to be controlled by a specification test, particularly when process controls can reliably ensure consistent quality.

This presentation advocates for a more flexible approach to vaccine specifications that acknowledges their unique characteristics while maintaining appropriate quality standards through risk-based assessment and comprehensive control strategies tailored to vaccine-specific attributes.

This work is funded by Sanofi.

The authors are Sanofi employees and may hold shares and/or stock options in the company.

Biosketch

Daisy Nie

Executive Director, Cell Therapy
Analytical Development and Operations
East Hanover, NJ 07936
Novartis Pharmaceuticals

Tel: +1 (973) 935 6612
E-mail: daisy.nie@novartis.com

Daisy Nie, Ph.D., is a recognized leader in the biopharmaceutical industry with extensive experience in the development, commercialization, and lifecycle management of biologic therapies, including therapeutic proteins and cell/gene therapy products. Currently, she serves as the Head of Cell Therapy Analytical Development and Operations at Novartis, where she leads a team responsible for developing analytical methods for release and characterization of Novartis Cell Therapy pipeline.

Prior to her role at Novartis, Daisy has held various key leadership positions at Thermo Fisher Scientific, Bristol Myers Squibb, and AstraZeneca. She has led various analytical CMC projects and control strategies supporting numerous clinical or commercial biologics programs.

With a Ph.D. in Microbiology and Immunology from Drexel University College of Medicine, Daisy combines her deep scientific knowledge with her strategic leadership skills to drive continuous improvement and operational excellence within the industry. Her expertise in analytical development, quality assurance, and regulatory submissions underscores her commitment to advancing patient-centric specifications and ensuring the highest standards of product quality and safety.

Abstract

Daisy Nie

Title: Strategies for Defining Specifications in Autologous Cell Therapy Products

In the evolving landscape of autologous cell therapy, the unique challenges of the product variability demand a new approach in setting product specifications. Unlike biologics and small molecules where variability stems from process and analytical variability, autologous cell therapy introduces significant donor variability, which becomes the primary source of overall variability. This presentation explores the fundamental differences between these therapeutic modalities and underscores the limitations of traditional specification frameworks when applied to autologous cell therapies.

A case study focusing on Vector Copy Number (VCN) will be presented, discussing the rationale behind setting two-sided versus one-sided specifications and evaluating the associated clinical safety risks in relation to specification limits.

By adopting a patient-centric approach to setting quality standards, this presentation will demonstrate how ensuring batch quality through deep understanding of product variability can lead to more flexible, safe, and efficacious autologous cell therapies, ultimately benefiting patients.

Biosketch

Shawn Novick

IABS Biotherapeutics Chair
Seattle, USA

E-mail: novicksl@hotmail.com

Shawn Novick has worked in the biotherapeutic field for over 30 years. She spent the first half of her career in analytical sciences, characterizing products for clinical and commercial approval including a number of cytokines, monoclonal antibodies, Fc-fusion proteins and protein conjugates. The second half of her career she was the Sr. Director of Quality Control at Seattle Genetics, where she built a Quality group from 4 to 50 analysts, working on an array of antibody drug conjugates. Shawn has contributed to a number of commercial products including Enbrel, Leukine, Bexxar, and Adcetris. She has written or edited multiple regulatory submissions and worked on successful CMC strategies for multiple products.

In addition to her day job, Shawn has organized and co-chaired workshops and conferences, including the annual WCBP conference held in Washington D.C. which brings together over 800 regulators and biotherapeutics professions for 3 days of close interaction. Shawn has published and presented on protein characterization and specification management and currently is consulting for biotechnology companies. She lives in Seattle, Washington.

Biosketch

Dr Horacio Pappa

Senior Director of the General Chapters Department, Global Science and Standards Division
United States Pharmacopeia
Rockville, MD 20852
USA

E-mail: hp@usp.org

Dr. Pappa has been with USP since 2003. He is currently the Senior Director of the General Chapters Department, Global Science division of the USP. He provides scientific leadership to a team of scientific liaisons responsible for the activities of seven different expert committees that cover the majority of the USP General Chapters. Horacio earned his Ph.D. in Pharmaceutical Chemistry from the University of Buenos Aires. He has authored many publications and peer-reviewed articles and is a frequent speaker and instructor on topics related to Chromatography and Validation. Prior to joining USP, he worked in the pharmaceutical industry in QA/QC. Horacio held the position of Assistant Professor of Quality Control in the Faculty of Pharmacy at Buenos Aires University, and Executive Secretary of the Argentine Pharmacopeia in the period 1997-2001. He is a Quality Engineer certified by the American Society for Quality.

Biosketch

Patrice Riou, PhD

Head of Global Analytical Strategy and Regulatory compliance
69280 Marcy l'Etoile, France
R&D/ Sanofi Vaccine

Tel: +33 (0)6 48 99 25 90
E-mail: patrice.riou@sanofi.com

PhD in Virology and Cellular Biology
21 years of experience within Sanofi Vaccine in Analytical R&D, involved in the Development and Validation of assays (Virology/Microbiology) and in the definition of analytical strategies for new vaccines. Since Jan 2020, head of a group of analytical and regulatory experts in charge of driving the New Vaccine Global Analytical Strategy and ensuring Regulatory Compliance.
Sanofi representative within EDQM expert group 15 for Human Vaccines and Sera, EPAA group for Rabies Vaccine in vivo potency assay replacement.

Abstract

Bénédicte Mouterde and Patrice Riou

Title: Vaccines are Biologicals with Unique Specificities

The enhanced approach/Quality by Design (QbD) represents the current preferred strategy for CMC process development and control strategy implementation for biologics, including vaccines.
However, vaccines possess unique characteristics that distinguish them from other biological products, particularly biotherapeutics and monoclonal antibodies. These specificities include their mechanism of action (immunogenicity as the basis for efficacy rather than a safety risk), route of administration, and dosing regimens.
Current regulatory frameworks and pharmacopoeial standards sometimes fail to account for these vaccine-specific attributes, applying requirements more suitable for other biologics and/or less applicable to the enhanced approach based on risk analysis and prior knowledge.
In this presentation, we will discuss concrete examples of CQAs that may be managed differently for vaccines as compared to other biologics, such as process-related impurities, subvisible particles, uniformity of dosage/content, and potency.
The implementation of QbD principles for vaccines requires recognition that while all specification tests address Critical Quality Attributes (CQAs), not all CQAs necessitate to be controlled by a specification test, particularly when process controls can reliably ensure consistent quality.
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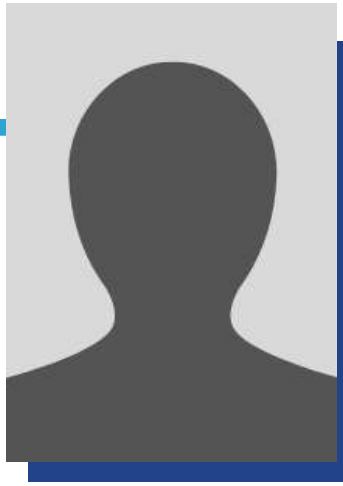
The authors are Sanofi employees and may hold shares and/or stock options in the company.

Biosketch



Akira Sakurai

Senior Scientist for Biopharmaceutical Quality
100-0013 Japan
Pharmaceuticals and Medical Devices Agency (PMDA)
Shin-Kasumigaseki Building, 3-3-2
Kasumigaseki, Chiyoda-ku, Tokyo

E-mail: sakurai-akira@pmda.go.jp

Akira Sakurai is Senior Scientist for Biopharmaceutical Quality at the Pharmaceuticals and Medical Devices Agency (PMDA). He manages the evaluation direction of biopharmaceutical quality including gene therapy products and the "Cartagena Act" regulation. He received Ph.D. in 2004 from Hokkaido University an M.S. degree in 2001, and a Bachelor of Pharmacy in 1998 from Kyoto University. His specialty is Virology, and he worked as a scientist at the University of Wisconsin-Madison, National Research Institute of Police Science, and Tokyo Metropolitan Institute of Medical Science until he joined PMDA in 2014. He was a member of the revision of ICH Q5A (Viral Safety Evaluation of Biotechnology Products) as deputy topic leader of MHLW/PMDA, Japan.

Biosketch

Kaushik Sarkar

Analytical Project Manager
Novo Nordisk
Måløv, Denmark

Tel: +45 34 48 47 54

E-mail: kugs@novonordisk.com

Kaushik Sarkar is a biotechnology professional with over 18 years of experience spanning antibody discovery, cell therapy development, and analytical sciences within the biopharma industry. He holds a PhD in GPCR antibody discovery and engineering from Imperial College London, completed in collaboration with UCB Pharma.

Kaushik's career began at UCB Pharma, where he gained extensive experience in monoclonal antibody discovery and development across both Research and CMC functions. During this tenure, he contributed to mechanism of action elucidation and served as patent author for Rozanolixizumab, a therapeutic antibody.

In 2019, Kaushik transitioned into the rapidly evolving cell therapy field, joining Adaptimmune as a CMC Senior Scientist and Team Lead. In this role, he worked on both autologous and allogeneic cell therapy platforms and made key contributions to the CMC assay development for Afami-cel, Adaptimmune's lead T-cell therapy candidate.

Currently, Kaushik serves with Novo Nordisk's Cell Therapy CMC department, where he manages analytical development programs and oversee method transfer to GMP production sites. His responsibilities include establishing drug substance and drug product specifications, ensuring seamless translation from development to QC and GMP manufacturing.

Abstract

Kaushik Sarkar

Title: Specification consideration for CGT products and role of analytics: Challenges and Opportunities

Cell therapy products present unique specification challenges that traditional regulatory frameworks like ICH Q6B were not designed to address. Unlike conventional biotechnology products, cell therapies are inherently heterogeneous, living entities with limited shelf-life and dynamic quality attributes that change during storage and transport. This presentation explores the specific analytical challenges faced in cell therapy development at Novo Nordisk.

Key specification challenges include detecting residual iPSCs using sensitive methods like digital droplet PCR, ensuring sterility within limited timeframes, and characterizing complex cell populations through multi-parameter flow cytometry. Traditional 14-day sterility testing is incompatible with short shelf-life products, necessitating rapid microbial methods such as BACT/ALERT and solid phase cytometry that can provide results within hours rather than weeks.

Advanced analytical tools including automated cell counting, flow cytometry, and ddPCR offer enhanced precision, sensitivity, and regulatory compliance while supporting comprehensive quality assessment. However, method validation under time constraints, sample stability issues, and re-source-intensive processes remain significant hurdles. The integration of these cutting-edge analytics is essential for establishing scientifically justified specifications and ensuring patient safety in cell therapy product development and release.

Biosketch

Yoji Sato, PhD

Deputy Director General
National Institute of Health Sciences,
Kanagawa, Japan

Tel: +81 44 270 6505
Fax: +81 44 270 6506
E-mail: yoji@nihs.go.jp

Dr Yoji Sato is Deputy Director General at the Japanese National Institute for Health Sciences (NIHS). As a graduate student at the University of Tokyo and a postdoctoral fellow at the University of Cincinnati, he conducted research in cardiovascular pharmacology and successfully established various transgenic mouse models to elucidate the mechanisms of cardiac excitation-contraction coupling and heart failure.

From 2014 to 2023, he served as Head of the Cell-Based Therapeutic Products Division at the NIHS, where he led a public-private partnership initiative (MEASURE Project) in Japan to validate a variety of test methods for evaluating the tumorigenicity of cell therapy products (CTPs) in collaboration with the Committee on Nonclinical Safety Evaluation of Pluripotent Stem Cell-Based Therapeutic Products of the Forum for Innovative Regenerative Medicine (FIRM-CoNCEPT). He also contributed to establishing Standards for Human Stem Cell Use in Research, which was published in June 2023, as a member of the Standards Initiative Steering Committee of the International Society for Stem Cell Research (ISSCR). In addition, he was a topic leader of the Expert/Implementation Working Group for ICH Q5A(R2). From April 2023 to March 2025, he served as Head of the Division of Drugs at the NIHS, responsible for quality assurance of small molecule drugs distributed in Japan, before being appointed Deputy Director of the NIHS in April 2025.

He continues to serve on the ISSCR's Clinical Best Practices Task Force on Pluripotent Stem Cells-Derived Products. He is also the Chair of the Database Committee of the Japanese Society for Regenerative Medicine, which provides the National Regenerative Medicine Database (NRMD), a national patient registry system for clinical and post-marketing research on CTPs, and has been a member of the Pharmaceutical Affairs Council of the Japanese Ministry of Health, Labour, and Welfare.

Biosketch

Hiroko Shibata, PhD

Section Chie

210-9501, Kawasaki, Japan

National Institute of Health Sciences

Address:3-25-26 Tonomachi

Tel: +81-44-270-6514

E-mail: h-shibata@nihs.go.jp

Dr. Hiroko Shibata is a Section Chief of Division of Biological Chemistry and Biologicals at the National Institute of Health Sciences, Japan. (NIHS). She is a member of the Japanese Pharmacopoeia Expert Committees for drug formulation and biologicals, and also an expert reviewing quality aspect of new drugs submitted for registration. She is also a topic leader of Q2(R2)/Q14 IWG.

Biosketch

Jayda Siggers, PhD

Senior Evaluator
Ottawa, Ontario, Canada
Biologic and Radiopharmaceutical Drugs Directorate (BRDD)/ Health Canada

E-mail: jayda.siggers@hc-sc.gc.ca

Dr. Jayda Siggers is a Senior Biologist/Evaluator in the Biotherapeutics Quality Divisions (BQD) of the Centre for Blood, Blood Products, and Biotherapeutics (CBBB) within the Biologics and Radiopharmaceutical Drugs Directorate (BRDD) at Health Canada. Since joining Health Canada in 2015, she has led the quality review of pre- and post-market drug submissions for biologic drugs.

Dr. Siggers holds a Master of Science in Toxicology from the University of Saskatchewan, a PhD in Immunology from the University of Copenhagen, and completed a postdoctoral fellowship in the Department of Biochemistry, Microbiology, and Immunology at the University of Ottawa.

She actively contributes to the advancement of biologics and regulatory standards. She represented Health Canada on the WHO drafting group for guidelines on monoclonal antibodies and related products and developed a training module for PAHO on the evaluation of therapeutic antibodies. She also co-chairs a PDA standard on analytical method transfer and comparability for biologics and leads an internal working group for the implementation of ICH Q2(R2)/Q14. Additionally, she contributed to the drafting of the new agile food and drug regulations and is currently revising Health Canada's biosimilars guidance.

Outside of work, Dr. Siggers enjoys cycling and can often be found chasing her family up hills and down mountains on one of her bikes.

Biosketch

Dean K. Smith, Ph.D.

Section Head and Senior Scientific Evaluator of Vaccines
Health Canada

Tel: +1 613-799-5689
E-mail: dean.smith@hc-sc.gc.ca

Advisor to the Director and Sr. Evaluator in the Center for Vaccines, Clinical Trials and Biostatistics at Health Canada. He has over 25-years of experience in research and regulatory science in support of innovation for vaccine development, manufacturing and quality control. He is active in the development / implementation of related guidance, and has a wide range of biologics-based scientific and regulatory experience from his Sr. Scientific Evaluator and management roles in Centre Divisions including Viral and Bacterial Vaccines, Hemostatic Agents & Blood Substitutes, as well as the Clinical Evaluation Division-COVID.

Representing Health Canada, he has contributed to WHO's smallpox and rabies vaccine guidance, the Extended Controlled Temperature Conditions (ECTC) guidance in support of innovative vaccine stability assessment for vaccination campaigns over the "last mile" with limited cold chain. Additionally, he contributed to WHO's R&D Blueprint International COVID-19 vaccine consultations during the pandemic, and since 2018 has been engaged in the regulatory / industry patient-centric harmonized specification exchanges in line with an assumed intent of ICH Q6B.

Biosketch

Masayo Takahashi, MD, PhD

President

650-0047 Kobe, Hyogo, Japan

Vision Care Inc.

Address: 2-1-8 Minatojima-minamimachi, Chuo-ku

Tel: +81-78-304-5260

E-mail: takahashisec@vision-care.jp

Masayo Takahashi is an ophthalmologist who completed her doctoral program (Visual Pathology) at the Graduate School of Medicine, Kyoto University. She served as the leader of the Laboratory for Retinal Regeneration, RIKEN Center for Biosystems Dynamics Research from 2006 to 2022, conducting groundbreaking clinical research using the world's first induced pluripotent stem (iPS) cells in 2014. In 2017, she collaborated with the city of Kobe to establish the "Kobe Eye Center." After leaving RIKEN in 2019, she became the CEO of Vision Care Inc. and subsequently founded two subsidiary companies dedicated to gene therapy development and cell therapy research.

Abstract

Masayo Takahashi

Title: Strategy for Retinal Cell Therapy

Developing retinal cell therapy with iPSC-derived retinal pigment epithelium (iPSC-RPE) cells has provided critical insights into robust manufacturing and patient-centric specification strategies. Our initial approach involved autologous iPSC-RPE transplantation, focusing on achieving high reproducibility across diverse patient samples and cell line generation processes. This led to the establishment of a robust and flexible manufacturing method, producing equivalent cell quality regardless of donor variability.

As we moved toward standardization, we evaluated different delivery formats. We learned that preclinical models were insufficient to determine optimal formulation. Clinical research involving actual patients was essential to identify the most effective and safe transplant formulation. This highlights a key regulatory consideration: cell-based products often differ in structure and function in vivo compared to their final released form. Specifications must reflect these clinical realities rather than rely solely on in vitro parameters.

Moreover, inefficient or overly rigid specification criteria can lead to unnecessary costs and delays. Regulators and developers must collaborate to design flexible, yet meaningful specifications grounded in their outcomes in patients and post-transplant behavior. Additionally, the rise of AI presents transformative opportunities in automating quality and process control, reducing human error, cost, and time across manufacturing stages.

This presentation will illustrate practical lessons from iPSC-RPE development and propose a forward-looking patient-oriented framework in regenerative medicine.

Biosketch

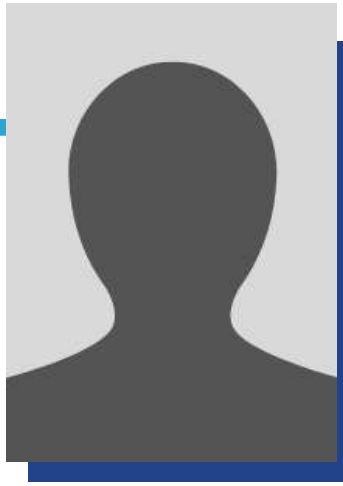
Maria Cecilia Tami, PhD

Head, US CMC Regulatory Policy
94080 South San Francisco, (CA), US
Genentech
1 DNA Way, Ste 258A

E-mail: tami.maria@gene.com

Dr. Maria Cecilia Tami received a M.Sc. in biology and a PhD in the field of molecular virology from the University of Buenos Aires, Argentina. Dr. Tami joined Genentech-Roche in 2020 as the Head of US Technical Regulatory Policy. In this capacity, she leads and executes CMC regulatory policy strategies and advocacy plans and external outreach in technical and regulatory CMC related topics. Before joining Genentech-Roche, she served for over 12 years in the Office of Biotechnology Products (OBP) in CDER, FDA and held positions of increasing responsibility. She performed and oversaw risk-based CMC/product quality assessments at all phases of product development. Dr. Tami is actively involved in ICH activities. While at FDA, she served as FDA Topic lead for the revision of ICH Q5A "Viral Safety Evaluation of Biotechnology Products". Currently, she is the deputy Topic Lead in the revision of ICH Q6, representing BIO and she is also the Deputy Topic Lead in the ICH Quality Discussion Groups (QDG), representing PhRMA. Dr. Tami is an active member of the CASSS community, an international non-profit scientific society. She serves as co-chair in several venues and is currently a member of the WCBP Scientific Organizing Committee. She is also a member of the Innovation and Quality (IQ) Consortium.

Biosketch



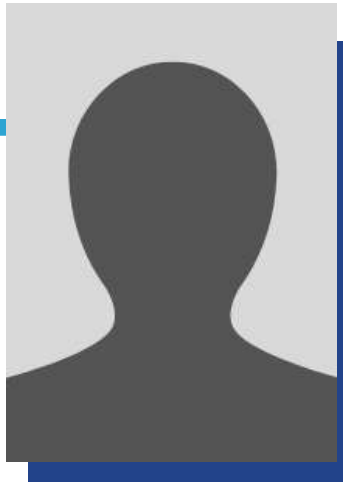
Bart van Montfort

Scientific Director/Lead Gene Therapy and ExPEC in Analytical Development
Leiden, South Holland, Netherlands
Johnson & Johnson Innovative Medicine

E-mail: bvanmont@its.jnj.com

Bart van Montfort is a seasoned professional in the biotechnology industry with over 20 years of experience specializing in analytical development and quality control. He holds an MSc in Molecular Sciences and a PhD in Biochemistry. Focused primarily on viral vectors for gene therapy and vaccines, Bart has played a pivotal role in several successful license applications, including the first licensed gene therapy product and the Janssen Covid Vaccine, and many clinical trial applications. His expertise lies in product specifications and their justifications, criticality analysis, integrated control strategy, and shelf-life claims.

Biosketch



Satoshi Yasuda, Ph.D.

Head of the Division of Cell-Based Therapeutic Products
210-9501 Kawasaki City, (Kanagawa), JAPAN
National Institute of Health Sciences (NIHS)
3-25-26 Tonomachi, Kawasaki-ku

E-mail: yasuda@nihs.go.jp

Satoshi Yasuda, Ph.D., is currently Head of the Division of Cell-Based Therapeutic Products, National Institute of Health Sciences (NIHS), Japan. He received his Ph.D. in Pharmaceutical Sciences from Nagoya City University. In his research career, he worked on biochemistry and cell biology as a postdoctoral fellow at the National Institute of Infectious Diseases and the University of Tennessee Health Science Center. After continuing his field of interest as an instructor at the Sapporo Medical University School of Medicine, he joined the Division of Cellular and Gene Therapy Products at NIHS in 2009 as a senior researcher. To date, he has been mainly involved in the development and validation of test methods to assess the safety and quality of cell-based therapeutic products derived from human pluripotent stem cells, particularly in terms of their tumorigenicity and differentiation bias. He participates in the ICH Cell and Gene Therapy Discussion Group as Deputy Topic Leader, the ISO/TC 276/SC 1 Committee, and an international consortium of the HESI CT-TRACS. He is also a visiting professor at Nagoya City University and a guest professor at Osaka University.

Biosketch

Takahiro Yamaguchi, Ph.D.

Assistant General Manager 410-
2321 Shizuoka, Japan Asahi
Kasei Pharma Corporation
Address: 632-1, Mifuku, Izunokuni

Tel: +81 558 76 8187
E-mail: yamaguchi.tdd@om.asahi-kasei.co.jp

Dr. Takahiro Yamaguchi is Assistant General Manager of Asahi Kasei Pharma Corporation. He has over 10 years of experiences as analytical scientist mainly for peptides and recombinant proteins including monoclonal antibodies, and experienced molecular characterization (e.g. LC/MS/MS), method development & validation, specification setting & quality control on investigational drug products. He has been involved in JPMA's biopharmaceutical committee and ICH Q6(R1) EWG as a topic leader from JPMA. He obtained Ph.D. of Pharmaceutical Sciences from Kyoto University

Abstract

Takahiro Yamaguchi

Title: Industry perspective on ICH Q6 revision and expectations for setting specifications based on enhanced approach

Since the establishment of ICH Q6B in 1999, the biopharmaceutical industry has experienced significant advancements in manufacturing and analytical technologies. Especially, the rise of monoclonal antibody drugs has been remarkable. Since monoclonal antibodies have common molecular structures and characteristics, manufacturers can streamline production, ultimately accelerating the development and availability of high-quality therapeutic antibodies. Analytical advancements, such as mass spectrometry, also allow for detailed analysis of quality attributes, leading to better product and process understanding and control of the manufacturing process, ensuring high-quality products.

In addition to technology advances in the biological field, new modalities such as antibody-drug conjugates, and cell and gene therapies, are emerging. To implement these advancements and reflect science- and risk-based approaches introduced from ICH Q8-11 guidelines, the revision of the ICH Q6A and Q6B guidelines has been adopted as a topic, and discussions have begun by the expert working group.
This presentation will delve into these technological developments, perspective on ICH Q6 revision, and discuss the expectations for setting specifications based on enhanced approach.