PDA Cell and Gene Pharmaceutical Products Conference 2025

Agenda

Tuesday, 18 November

Japan Standard Time Standard Time (UTC +9:00)

09:30 – 10:00	**Registration**	
10:00 – 10:15	**Opening Session**	
	10:00 – 10:15	**Opening Remarks** • **Shingo Sakurai** , Professor, *Tokyo University of Science, Laboratory of Pharmaceutical Quality Design and GMP*
10:15 – 11:25	**Current and Future Regulations (Part 1)**	
	10:15 – 10:50	**Current situation regarding regenerative medicine products: from the perspective of the Pharmaceuticals and Medical Devices Act** Regarding regenerative medicine in Japan, the 2013 amendment to the Pharmaceuticals and Medical Devices Act established a system that allows for conditional and time-limited approval for a new category of "regenerative medicine products," aiming to expedite their practical application. Furthermore, this year's amendment to the Pharmaceuticals and Medical Devices Act allows for the provision of autologous products to patients even if they do not meet the standards, provided that certain conditions, such as medical necessity and safety, are met. We are continuing to work on responding to the characteristics of each product. It is expected that groundbreaking regenerative medicine products will be put to practical use in the future, and I would like to share with you my understanding of the current situation, challenges, and direction from the Ministry of Health, Labour and Welfare. • **Yumiko Nomura** , Director of the Medical Devices Review and Licensing Division, *Ministry of Health, Labor and Welfare*
	10:50 – 11:25	**Key Quality Issues in the Review of Regenerative Medical Products** Over a decade has passed since the Pharmaceuticals and Medical Devices Act (PMD Act) was amended to establish a dedicated chapter for regenerative medical products, and more than twenty such products have now obtained marketing authorization in Japan. Quality review of these products covers a broad spectrum of elements, including product specifications, shelf-life setting, risk management of adventitious agents, and process validation. In this presentation, I will provide an overview of key quality considerations, illustrated with examples of inquiries raised during the review. In addition, I will introduce the “Guidance on the Preparation of Application Dossiers and Summary Descriptions for Regenerative Medical Products,” made available in March 2025, as a useful reference for preparing quality-related documents for marketing authorization applications. • **Ayaka Okamoto Ph.D.**, , *PMDA Regenerative Medicine Products Review Division*
	Lunch	
		Körber Demo Session- Introduction and Future Outlook of Electronic Records in Regenerative Medicine Facilities Cell and Gene Therapy (C&GT) is a cutting-edge treatment that has made significant progress in recent years. It is attention as a personalized option for intractable diseases, including cancer. Especially for autologous C&GT products, advanced manufacturing technologies, clean facilities, and strict quality assurance are required throughout the process—from collection to processing, culturing, and reinfusion. As each product is manufactured for each patient, ensuring data integrity and traceability is essential. Meanwhile, C&GT is still an evolving field. Compared to conventional biologics, standardization and

11:25 – 12:25	11:35 – 11:50	regulatory frameworks are still under development. Regulatory compliance, like the manufacturing system itself, requires flexible and ongoing revisions. In this presentation, we will use an ongoing project conducted by Teijin Regenerative Medicine Co., Ltd., a CDMO specializing in regenerative medicine products, and Körber Japan, which provides PAS-X MES(Manufacturing Execution System), as an example to introduce efforts in implementing electronic records for CAR-T cell product manufacturing and discuss future perspectives. It will address challenges and solutions related to the standardization of manufacturing processes, reducing human error and realizing strict quality control. • **Nanami Taniguchi** , MES Consultant, *Körber Japan K.K. Pharma Software Division*
	Current and Future Regulations (Part 2)	
12:25 – 14:25	12:25 – 13:15	**Draft Revision of the Japanese Standards for Biological Raw Materials** In order to ensure the quality, efficacy and safety of biological products including cell/gene therapy products, it is necessary to ensure the quality and safety of the raw materials used in the manufacture of these products. Therefore, in Japan, raw materials derived from humans and other living organisms (excluding plants) used in the manufacture of biological products which are manufactured and distributed under the Pharmaceuticals and Medical Devices Act, must comply with the Standards for Biological Raw Materials (SBRM). However, scientific knowledge on the safety of raw materials is being accumulated. In addition, there have been reported cases where the differences between Japan's SBRM and the standards for biological raw materials required overseas have caused significant delays in the domestic introduction of overseas products and hindered the overseas distribution of domestically developed products. Therefore, a working group consisting of experts from academia, regulatory authorities, regulatory science, and infectious diseases was established, and discussions were carried out while incorporating opinions and up-to-date information from stakeholders from industries and regulatory authorities. In this session, I would like to introduce the direction of the SBRM revision proposed by the working group. • **Yoji Sato Ph.D.**, Deputy Director General, *National Institute of Health Sciences, Kanagawa, Japan*
	13:15 – 13:50	**An Overview of the Advanced Regenerative Medicine Regulation and Industry in Korea** Korean government has made substantial investments in R&D and policy support, particularly focusing on serious and rare diseases. This prioritization is intended to foster innovation and accelerate the development of new therapies, thus addressing critical health issues more effectively. 'The Advanced Regenerative Bio Act' was enacted in 2019 to improve patient accessibility of advanced regenerative medicine and to support the development of advanced biopharmaceuticals. This act aims to create a robust legal and regulatory framework to support the growth and innovation in this sector. In February 2024, the Advanced Regenerative Bio Act was amended to increase accessibility of patients and safety of advanced regenerative medicine. By addressing both opportunities and challenges of the revised Act, stakeholders can work towards a future where innovative therapies are developed efficiently and safely, ultimately benefiting patients with serious and rare medical conditions. This presentation explores the current status of Korea's regulatory system of advanced regenerative medicine and industrial ecosystem. • **So-Ra Park M.D., Ph.D.**, President & Founder, Regenerative Medicine Acceleration Foundation, *Professor, College of Medicine, Inha University*
	13:50 – 14:25	**Taiwan ATMP ecosystem and enabling CDMO** • **David Y.H. Chang, Ph.D.** , CEO, *Taiwan Bio-Manufacturing Corporation (TBMC)*
14:25 – 15:10	**Coffee Break**	
	Initiatives related to ATMP at PDA Headquarters	
		PDA Headquarters' Initiatives Regarding ATMP The PDA's four advisory boards (ATMP AB, Biopharmaceutical AB, Regulatory Affairs and Quality AB, and Science AB) provide the PDA Board of Directors with scientific and technical expertise. They also guide PDA's scientific, regulatory and technical initiatives, collaborating closely with PDA Interest Groups. The PDA ATMP AB was established in 2020. It provides guidance and sets strategic directions on cell,

15:10 – 16:55	15:10 – 15:45	gene, tissue, and other novel therapeutic modalities. In addition to commenting on ATMP guidelines from the FDA, EMA, USP, EC and WHO, we publish 'Points to Consider' documents and articles on critical topics such as raw and ancillary materials, drug manufacturing, quality systems, product and process comparability, and analytical methods (e.g. PtC No. 11 – Development, Classification, Manufacture, Control and Testing of Plasmids and Vectors Used in ATMP Production; PtC for Microbial Control in ATMP Manufacturing; PtC for Materials in ATMP Manufacturing; PtC for ATMP Facilities; and PtC for QC Sequencing). • **Friedrich von Wintzingerode PhD**, Director, Microbiology and QC Individualized and Cell Therapy, *Genentech, a Member of the Roche Group*
	15:45 – 16:20	**Points to Consider(PtC) No. 11: Development, Classification, Manufacture, Control, and Testing of Plasmids and Vectors Used in ATMP Production** • **Darius Pillsbury** , Senior Consultant, *ValSource, Inc.*
	16:20 – 15:55	**Introduction to Points to Consider Regarding ATMP Raw Material Management** • **Takehiro Okumura Ph.D.**, General Manager, Alloy Therapeutics Japan, *Head of Quality, Alloy Cell Therapies*
17:00 – 19:00	**Social Gathering**	

Wednesday, 19 November

Japan Standard Time Standard Time (UTC +9:00)

08:30 – 09:00	**Registration**	
09:00 – 10:10	**Product Development (Part 1)**	
	09:00 – 09:35	**Development of Regenerative Medicine Product "Akuugo"** Akuugo® Intracerebral Transplant Injection (hereinafter "Akuugo") is a designated regenerative medicine product (human somatic stem cell processed product) created by SanBio Co., Ltd., and is a combination product consisting of the main component (main component cell: Vandefitem cell) and the sub-components (dedicated preparation solution and dedicated administration device set) required for transplantation into the brain. This product obtained manufacturing and sales approval (conditional and time-limited approval) on July 31, 2024 for the efficacy, effect, or performance of "improving chronic motor paralysis associated with traumatic brain injury (hereinafter "TBI")." Akuugo is the first allogeneic human bone marrow-derived processed mesenchymal stem cell approved for manufacture and sale in Japan that acts directly on damaged brain tissue. It is a new therapeutic drug used to improve chronic motor paralysis in TBI patients with motor dysfunction. In addition, this product is processed and manufactured using bone marrow-derived cells from healthy adults as raw materials, making it possible to stably supply the amount required for treatment, and there is no direct or indirect burden (treatment timing can be adjusted) on the patient. On the other hand, as a manufacturer and distributor, we strive to provide a stable supply of our products, and since human cells are used as an important raw material, even though they are allogeneic, the variation from the raw material is not small, and furthermore, by introducing the NICD gene, the variation in the final yield was not small. Furthermore, unlike chemical products or biopharmaceuticals, analyzing the product's quality characteristics, which are necessary for obtaining manufacturing and marketing approval, is not easy, just like with other regenerative medicine products. Here, we would like to share the development history of Akuugo up to the point of obtaining pharmaceutical approval, as well as our experience with the product's quality characteristics and equivalence assessment, in the hope that it will be of help to those struggling with the development of regenerative medicine products. • **Kazumi Sawaguchi** , Head of Quality Assurance and Regulatory Affairs Division, *SANBIO*
		Development and Post-marketing Issues of Japan's First Allogeneic Regenerative Medicine Product "TEMCELL ® HS Injection In September 2015, JCR Pharmaceuticals., Ltd. obtained manufacturing and marketing approval from the Ministry of Health, Labour and Welfare for allogeneic mesenchymal stem cell product, TEMCELL® HS Injection, for the treatment of acute graft-versus-host disease (acute GVHD) following hematopoietic stem cell transplantation. This product is the first in Japan to receive approval as allogeneic regenerative

	09:35 – 10:10	medicine product, and the first mesenchymal stem cell product in Japan, the United States and Europe. The product was launched in February 2016, and about 9 years after its launch, more than 1,000 patients have already been treated with TEMCELL® HS Injection. Currently, we are systematically and continuously producing and shipping TEMCELL® HS Injection in order to stably supply the product as a marketed product. In regenerative medicine products, it is difficult to identify and control the effect of variable factors such as cell source on the quality, and especially in many cases, it is difficult to sufficiently verify the characteristics of the product with only a relatively small number of lots performed in the development phase. Therefore, as the number of production lots increases after the market launch, issues that were not anticipated at development phase have gradually become apparent. In this presentation, we would like to share the situation and issues after the market launch of TEMCELL® HS Injection. • **Kiwamu Imagawa** , , *JCR Pharmaceuticals Co., Ltd. / AlliedCel Corporation*
10:10 – 10:20	**Break**	
10:20 – 11:30	**Product Development (Part 2)**	
	10:20 – 10:55	**The Use of Rapid Microbiological Methods (RMM) for Sterility Testing in ATMPs Manufacturing** In recent years, the use of Advanced Therapy Medicinal Products (ATMPs) has increased. The significant difference between ATMPs and conventional sterile pharmaceuticals is that ATMPs often use living cells as raw materials and in many cases, their time to administration to patient is very short; sometimes just a few days. For such short shelf life sterile products, it is ideal to complete the release tests (sterility tests) before administration to patients. However, conventional culture-based sterility tests take 14 days to obtain results. Rapid sterility testing is therefore critical to meeting such requirements. This section introduces the regulatory trends and current status of Rapid Microbiological Methods (RMM) for ATMPs, and further discusses the study and progress on the use of "High-Sensitivity ATP Method," an effective RMM for sterility testing of ATMPs. Specifically, this method quantifies ATP derived from microorganisms with high sensitivity, enabling sterility test results to be obtained within several hours to a few days, and its potential is also discussed. • **Yasuhito Ikematsu** , Associate Professor, Graduate School of Engineering at The University of Osaka, *Chair of Aseptic Product GMP Committee, Japan PDA Pharmaceutical Society*
	10:55 – 11:30	**Considerations for Aseptic Cleaning Based on Visualization of Residual Stuff in Grade A Areas of ATMPs Manufacturing** In ATMPs manufacturing, maintaining the environment through effective cleaning is crucial. This study developed and analyzed a quantitative evaluation method for the residue remaining in Grade A areas, which are critical for aseptic operations, using a model case of aseptic operation. ATP has been used as an indicator for biological contamination in food manufacturing, and we attempted to apply this method. Specifically, we measured the amount of ATP remaining on the surface of the biosafety cabinet before and after cleaning, compared the residue removal capabilities of different decontaminants, and assessed the performance of the operators. This section introduces the study and progress on the potential of using ATP as a new indicator for environmental management in ATMPs manufacturing and for the rapid confirmation of environmental status. • **Atsushi Kaneseki** , , *ROHTO Pharmaceutical Co., Ltd., Regenerative Medicine Research Planning Division / Japan PDA Pharmaceutical Society, Aseptic Product GMP Committee*
11:30 – 12:30	**Lunch**	
	Facility design & CCS	
	12:30 – 13:05	**A training on the Facility Design for ATMPs for inspector** The presentation is a summary of the presentation from Richard Denk during the 27th PIC/s Expert Circle Meeting on Human Blood, Tissues, Cells and Advanced Therapy Medicinal Products in August 2024 in Kuala Lumpur Malaysia he was invited. Quality Risk Management QRM and Contamination Control Strategies CCS and how do they apply for AMPS? More and more ATMPs are commercialized and the GMP Compliance manufacturing plays an important role. The Presentation covers the Contamination Control Strategy CCS for Barrier as closed Systems like Single Use Systems or Isolators as well as open systems like Bio Safety Cabinets. Including how to protect the pharmaceutical product from external contamination during the whole process as the most of the ATMPs can`t be terminal sterilized or sterilized by filtration and for that reason Engineering Control with Barriers and Facility/Room design should meet current GMP Standards. Furthermore, working with Viral Vectors or Autologous Products, Cross contamination prevention should be considered

as well as the protection of the operators.

- **Richard Denk** , Senior Consultant Aseptic Processing, *SKAN AG*

12:30 – 13:40

Setup of a Contamination Control Strategy for ATMPs using the Hazard Analysis Critical Control Point (HACCP) Methodology

A Contamination Control Strategy (CCS) is a strategy that focuses on how to prevent contaminations with microorganisms, particles and pyrogens and manages to medicinal product quality and patient safety within a GMP facility. It should reflect on all proactive and retrospective data within a sterile and/or aseptic and/or non-sterile manufacturing environment, to identify all sources of contamination and associated hazards and/or control measures. It should outline associated Quality Risk Assessments (QRAs), Critical Control Points (CCPs) and suggest necessary control measures. An effective way to perform QRA for CCS is adopting the Hazard Analysis Critical Control Point (HACCP) methodology, a proactive risk assessment tool. This tool can be ideally used to monitor all CCPs associated with various sources.

13:05 – 13:40

It can also be used to monitor CCPs associated with manufacturing of Advanced Therapy Medicinal Products (ATMPs). These drugs, which are administered to the human body as an injectable medicine need to be sterile and therefore need to meet regulatory requirements as stated in EudraLex Volume – Part IV “Guidelines on Good Manufacturing Practice specific to Advanced Therapy Medicinal Products” and EudraLex Volume 4 Annex 1 “Manufacture of Sterile Products”. Both Part IV and Annex 1 expect that a CCS is based Quality Risk Management (QRM) principles across their manufacturing facilities to manage any potential contamination risks. The constructed CCS allows the manufacturer to identify whether all included sources of contamination are under control and, if not, which mitigatory actions need to be performed. All current states are reflected by using the HACCP methodology as a traffic light color to reflect the level of residual risk, thereby providing a simple and clear visual representation of the current contamination control and microbial state of the manufacturing site.

- **Ruben van der Galiën Dr.**, Qualified Person | Quality Assurance Professional | Pharmacist, *GE Healthcare*

Issues Surrounding Containers (Part 1)

13:40 – 14:50

The Critical Role of Container Closure Integrity in Cell & Gene Therapy (CGT) and Biologic Packaging

Pharmaceuticals developed for Cell & Gene Therapy (CGT) introduce unique challenges to the industry due to their compositional characteristics along with stringent requirements for sterility and efficacy. These products are typically administered via pre-filled syringes, vials, or cartridges and are frequently subjected to storage conditions well below ambient temperatures. The preservation of sterility, potency and efficacy hinges on meticulous consideration of package component compatibility and optimization of processes throughout the fill and finishing processes.

In the context of cell and gene therapies, where the integrity of biological materials is paramount, container closure integrity testing (CCIT) plays a vital role in upholding regulatory standards, maintaining critical quality attributes, and guaranteeing patient safety. Ensuring the integrity of the container closure system is instrumental to safeguarding the risks of contamination or degradation, ensuring that the therapeutic products remain safe and effective throughout their shelf life.

Together we will focus on understanding which test methods to deploy across each stage of the product lifecycle and why inspection and integrity testing is necessary at certain stages.

Key Takeaways:
·Factor to consider when inspection CCI of CGT Pharmaceuticals / Biologics
·Which test methods are available and how to match applications to product-packages
·How to scale & automate test methods to increase inspection in a rapid and deterministic manner
·Regulatory requirements, guidance and recommendations driving integrity testing & inspection (EU Annex 1, USP 1207, PIC/S, ICH, FDA, PDA TR86)

Attendees will leave with the framework and tools to better establish test methodology and deploy quantitative test results to improve quality management for high-risk package applications.

- **Noba Ebaid** , Director of Sales, Americas & Asia-Pacific, *PTI – Packaging Technologies & Inspection*

Until it reaches the patient: The reality of containers, packaging, and transportation of regenerative medicine products

Since regenerative medicine products contain living cells, there are several challenges in terms of containers, packaging, and transportation that are different from pharmaceuticals in order to deliver the

	14:15 – 14:50	products to patients while maintaining their quality. Our company (Japan Tissue Engineering Co., Ltd.) has manufactured and sold five regenerative medicine products, namely Jace, Jack, Nepic, Ocular, and Jasmine, and has responded to each product's characteristics. Tissue structures constructed by culture are more fragile than real tissues and require careful handling. In particular, since our products involve transplant procedures, we design packaging containers in consultation with doctors, taking into consideration how they will be handled at medical institutions. In addition, products with three-dimensional structures are difficult to freeze, and the period during which the quality of living cells can be maintained is limited, so we set storage temperature zones according to the characteristics of each product and aim to maximize storage time. Furthermore, in order to provide regenerative medicine products stably, it is essential to develop transportation containers with excellent temperature retention performance, which enables the stable supply of irreplaceable products. In this presentation, based on the examples of these five products, we will introduce the challenges of containers, packaging, and transportation for regenerative medicine products and specific responses to them. • **Takahiro Ogasawara** , Director of Product Development, *Japan Tissue Engineering Co., Ltd. (J-TEC)*
14:50 – 15:20	**Coffee Break**	
15:20 – 17:00	**Issues Surrounding Containers (Part 2)**	
	15:20 – 16:10	**Aligned Control Strategy Proportions and Staging Opportunities—Concept and Execution Roadmap for an Enhanced and Integrated Analytical Control Strategy for Autologous Cell Therapies** • **Stephan O. Krause Ph.D.**, Executive Director Analytical Strategy, *BMS Cell Therapies*
	16:10 – 17:00	**Development of smart cell manufacturing based on the QbD approach** In the Quality by Design (QbD) approach to cell manufacturing, a thorough understanding of cell properties, environmental properties, and operational properties—along with risk assessment at the research stage—critically influences the subsequent design of the manufacturing process, ultimately leading to actual production. Building on the knowledge obtained during the research phase, the process development stage involves the timely and appropriate implementation of Process Analytical Technology (PAT) to evaluate the criticality of process parameters (PPs), identify critical process parameters (CPPs), and define the design space (DS). To realize smart manufacturing in cell processing, it is essential to develop enabling technologies across all stages, including process monitoring, digital data accumulation, criticality assessment, process optimization, and predictive modeling. We will introduce our integrated approach and share representative tools we have developed, with the aim of fostering discussion. • **Masahiro Kino-oka** , Professor, *Department of Biotechnology, Graduate School of Engineering, Osaka University & Research Base for Cell Manufacutrability, Graduate School of Engineering, Osaka University*
17:00 – 17:10	**Summary & Closing Remarks** **Richard Denk** , Senior Consultant Aseptic Processing, *SKAN AG*	