8th DIA Cell and Gene Therapy Products Symposium in Japan

"Development of Cell and Gene Therapy Products in the New Growth Phase"

January 15-16, 2024
Hybrid | Nihonbashi Life Science HUB & Virtual

In previous *Cell and Gene Therapy Products* symposia, we have discussed various challenges in the "introductory stage" following enactment of the Regenerative Medicine Promotion Act, such as "practical use" that turns a new scientific or technological concept to a therapeutic application, "industrialization" that enables reliable and stable delivery of therapeutic products to patients, and "mastering / implementation" that fulfills healthcare professionals' requirements in use of these new therapeutic modalities. During the "introductory" stage, the basic infrastructures for research and development have been established, in addition to the regulatory infrastructure which enabled the continued innovative activities to translate seeds of technologies to new therapeutic approaches. As a consequence, the number of disease areas in which cell and gene therapy products are considered one of the treatment options has increased.

As the milestone, tenth anniversary since the enactment of the Regenerative Medicine Promotion Act, we organized last year's 2022 *Symposium* to make a further leap forward, look back on our initial passion, and review the progress of product development in the past decade. By looking back over the decade of the "introductory stage," many participants realized something new or gained a stronger desire to enhance the efficacy and safety of these product. Organizers assumed this year's Symposium as the "first step" of the upcoming "growing phase," which provides growth opportunities for all the persons concerned.

This 2023 *Symposium* will feature the Director of Bio-Industry Division, Ministry of Economy, Trade and Industry Hirokazu Shimoda's keynote speech on the grand design of regenerative medical products, and VC Cell Therapy President Masayo Takahashi's lecture on her experience in promoting regenerative medical products as both a clinician and a business person. In addition, we plan a special discussion on the themes of industrialization of regenerative medical products and establishment of the next generation's business model with panelists who have led regenerative medical products in Japan from the industry side.

Sessions will include various topics ranging from development to post-marketing based on recent trends. The Symposium will also address a topic that will be covered at the *DIA Japan Annual Meeting* by deepening discussions on how to develop the post-marketing efficacy and safety evaluation plan considering the data obtained during development, based on the draft guideline of the MHLW research group. In the international standards session that was highly evaluated last year, we will discuss promoting the use of international standards through the certification system, diversifying modalities, and expanding to application fields. Post-marketing sessions will address the quality and clinical perspectives. The quality session will discuss how to utilize the post-approval change management (PACMP) system to smoothly proceed with regulatory procedures after approval. The clinical session will discuss the involvement of disease experts in collecting and evaluating efficacy and safety data in post-marketing clinical practice and cooperation among industry, government, and academia. Additional educational lectures will explore development of in vivo gene therapies with tissue / cell specificity as well as the concept of ensuring viral safety.

The stage for the development of cell and gene therapy products is changing from the "introductory stage" to the "growth phase". While the stage is changing, this Symposium will continue to provide the platform where industry, government, and academia share issues and discuss constructive solutions.

We plan to hold a hybrid meeting. Drinks and snacks will be provided at the venue during networking breaks. In addition to a service for downloading presentation materials (PDF files), we are also planning a service for distributing the recordings of each session and keynote speech* so you can make full use of the Symposium contents according to your needs. We look forward to seeing as many participants as possible this year.

* Please note that there may be restrictions on recording and sharing materials based on the content and nature of each session or lecture.

Simultaneous Translation Available

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10:00-10:15 WELCOME AND OPENING REMARKS

Shogo Nakamori, MSc,RPh, MBA
DIA Japan

Kiyoshi Okada,MD, PhD
Program Chair
Osaka University Hospital, Osaka University

10:15-10:45 KEYNOTE ADDRESS 1

SESSION CHAIR

Kiyoshi Okada,MD, PhD
Osaka University Hospital, Osaka University

TBD

Hirokazu Shimoda
Director, Bio-Industry Division, Commerce and Service Industry Policy Group, Commerce and Information Policy Bureau, Ministry of Economy, Trade and Industry (METI)

10:45-11:25 KEYNOTE ADDRESS 2

SESSION CHAIR

Kiyoshi Okada,MD, PhD
Osaka University Hospital, Osaka University

TBD

Masayo Takahashi, MD, PhD
President, VCCT Inc.

11:25-11:40 SHORT BREAK

11:40-12:40 SPECIAL PANEL DISCUSSION

Accelerating the Development and Access of Regenerative Medicine

SESSION CHAIR

Kiyoshi Okada,MD, PhD
Osaka University Hospital, Osaka University

Regenerative medicine, which has the potential to cure diseases that were previously considered incurable, is regulated by two laws in Japan: the Pharmaceutical and Medical Devices Act (PMD Act) and the Act on the Safety of Regenerative Medicine (Safety Act).

According to the PMD Act, regulated regenerative medicine is covered by public health insurance, but the regulatory approval process can be lengthy and complex, leading to delays in making regenerative medicine available to patients. According to the Safety Act, regulated regenerative medicine is generally provided on a fee-for-service basis, which can be expensive for patients.

This session will discuss how to make regenerative medicine more accessible to patients, including streamlining the approval process and expanding public health insurance coverage, in Japan.

Panelist

Hirokazu Shimoda
Director, Bio-Industry Division, Commerce and Service Industry Policy Group, Commerce and Information Policy Bureau, Ministry of Economy, Trade and Industry (METI)

Masayo Takahashi, MD, PhD
President, VCCT Inc.

Masashi Ogo
Director, Regenerative Medicine Research & Business Development Department, Shiseido Company, Limited

Yoshitsugu Shitaka, PhD
Representative Director, Chairperson, Forum for Innovative Regenerative Medicine / Chief Scientific Officer, Astellas Pharma Inc.

12:40-13:55 LUNCH BREAK

13:55-15:35 SESSION 1

International Standards : Promoting Utilization and Responding to Diversification

SESSION CO-CHAIRS

Shinichi Noda, PhD
Deputy Review Director, Office of Cellular and Tissue-based Products, Pharmaceuticals and Medical Devices Agency(PMDA)

Ikuo Kawauchi, MS
International Standards Promotion Office, Intellectual Property Div.
FUFIFILM Holdings Corporation

This session will outline international standards in the field of regenerative medicine to be developed and published by ISO/TC 276, focusing on the progress made since last symposium. In addition, certification systems, the measures to actively utilize these standards, for products and services of supporting industries and ancillary materials such as culture media and reagents, manufacturing equipment, and transportation services will be explained. Further, we will explain the status of standardization for gene therapy and MPS (Micropysiological System) as standards development responds to the diversification of modalities and the application of regenerative medicine technology, respectively.

Panel discussion will focus on expectations for the certification system and the expected effects on standards related to gene therapy and MPS.

International Standard in the Field of Regenerative Medicine

Tetsunori Matsumoto
Manager, MIRAI Technology Institute, Shiseido CO., LTD.

Implementation of Standards Through Conformity Assessment

Ikuo Kawauchi, MS
International Standards Promotion Office, Intellectual Property Div.
FUFIFILM Holdings Corporation

Responding to Diversification of Modalities : Status of Standard Development Related to Gene Therapy

Hiroshi Yoshida, PhD
Chief Engineer, Life & Medical Systems Product Division, Hitachi High-Tech Corporation

Expansion to Regenerative Medicine Application Fields : Microphysiological System

Yuzuru Ito, PhD
Professor, Faculty of Life and Environmental sciences, University of Tsukuba

Panel Discussion

All Session Speakers

15:35-15:55 COFFEE BREAK

15:55-17:45 SESSION 2

Post-Marketing Clinical Practice of Cell and Gene Products

SESSION CO-CHAIRS

Masafumi Onodera, MD, PhD
Director, Gene & Cell Therapy Promotion Center, NCCHD

Yoshie Tsurumaki, RPh
Senior Medical Lead, Novartis Pharma K.K.

The data available from clinical trials of regenerative medical products (RMPs) are often limited, and the mode of action for cellular therapy are sometimes not fully understood. As a result, it is important to continue to evaluate the safety and effectiveness of RMPs post-market. This symposium will bring together experts and professional associations from various disease areas to share their experiences and insights to collaboratively discuss how to develop a more effective post-market evaluation system for RMPs in the future.

Specific topics to be discussed: a) The challenges of post-market evaluation for RMPs; b) Current approaches to post-market evaluation; and c) Future directions for post-market evaluation.

Expected outcomes: a) Increased understanding of the challenges and opportunities of post-market evaluation for RMPs; and b) Development of a roadmap for improving the post-market evaluation system for RMPs.

Current Status of Collection and Evaluation of Post-Marketing Safety Information on Regenerative Medical Products

Masaki Kasai, PhD
Assistant Investigator, Pharmaceuticals Safety Division II, Pharmaceuticals and Medical Devices Agency (PMDA)

TBD

Yoshiko Atsuta, MD, PhD
Director, Japanese Data Center for Hematopoietic Cell Transplantation

TBD

Tomohiro Morio, MD, PhD
Vice president, Tokyo Medical and Dental University

TBD

Hirofumi Komaki, MD, PhD
Director of the Translational Medical Center, National Cener of Neurology and Psychiatry

Panel Discussion

All Session Speakers

17:45-18:00 SHORT BREAK

18:00-19:00 NETWORKING

9:30-9:45 OPENING - DAY2

Development Status and Issues of Gene Therapy Products

Masafumi Onodera, MD, PhD
Director, Gene & Cell Therapy Promotion Center, NCCHD

9:45-11:45 SESSION 3

Utilization of the J-PACMP System for Cell and Gene Therapy Products

SESSION CO-CHAIRS

Teruyo Arato, PhD
Professor, Clinical Research and Medical Innovation Center, Hokkaido University Hospital

Ryutaro Hirasawa, PhD
CMC Regulatory Affairs, Manager, Daiichi Sankyo Co., Ltd.

If there is a poor experience of manufacturing and quality control in development of cell and gene therapy products, it is conceivable that the product is launched without sufficient consideration regarding CMC development in the context of short-term clinical development focusing on earlier approval. Therefore, even in the post-approval phase, it is reasonable to expect that regulatory change procedures are required to improve the manufacturing process, whether based on clinical data or scale-up due to increased demand. Regarding post-approval change management in Japan, the J-PACMP system based on ICH Q12 has been implemented, driven by amendment of the Pharmaceuticals and Medical Devices Law in 2019.

This session will discuss how to effectively utilize the J-PACMP system for post-approval change management of cell and gene therapy products, from the industry and health authority perspectives.

TBD

Jun Matsumoto, PhD
Review director, Department of Cellular and tissue derived products, Pharmaceuticals and Medical Devices Agency (PMDA)

TBD

Yuriko Ishii, PhD
CMC Reguratory Affairs Department, Associate Director, Daiichi Sankyo Co., Ltd

Perspectives to be Considered for Utilizing the Japanese PACMP for Regenerative Medical Products

Keigo Kawabe, PhD
Sumitomo Pharma Co., Ltd.

Change Control for Cell Therapy Product : Utilization of PACMP and Future Perspective

Naoyuki Hanada
Novartis Pharma K.K.

Panel Discussion

All Session Speakers

11:45-12:00 SHORT BREAK

12:00-12:30 EDUCATIONAL LECTURE 1

SESSION CHAIR

Sumimasa Nagai, MD, PhD
Professor, Department of Medical Development, Kyoto University Hospital, Kyoto University

The Pharmaceuticals and Medical Devices Agency (PMDA) has established a Scientific Committee independent from its operations, which has published 17 reports regarding points to consider when reviewing products that apply cutting-edge science and technology in collaboration with specialized committees of external experts. Recent Committees have focused on gene therapy products, using genome editing technology, with extremely high potential for practical application.

This session will discuss the expectations and challenges of these products by explaining the Expert Subcommittee report on Genome Editing published in 2020 and introducing the current Committee for “Target-specific in vivo gene therapy products.”

PMDA Scientific Committee Recommendations for Gene and Cell Therapy Products

Teruhide Yamaguchi, PhD
Director, Institute of advance Medical and Engineering technology, Kanazawa Institute of Technology

Akihiro Kume, MD, PhD
Professor, Center for Clinical Investigation, Jichi Medical University Hospital

12:30-13:50 LUNCH BREAK

13:50-15:50 SESSION 4

Direction to Review of Regenerative Medicine Products - What we should/can do to obtain "True approval" –

SESSION CO-CHAIRS

Kiyoshi Okada,MD, PhD
Osaka University Hospital, Osaka University

Sumimasa Nagai, MD, PhD
Professor, Department of Medical Development, Kyoto University Hospital, Kyoto University

In the 10 years since the revision of the PMD Act, the number of approved regenerative medical products has increased and the concept of regulatory review has matured. Until now, industry, academia, and government have held various discussions to promote development of regenerative medicine products; one of the initiatives growing from these discussions is for MHLW's research group to prepare draft guidelines for post-marketing evaluation after time-limited approval, during this fiscal year.

This session will explain the concept of this guideline and also discuss what we should/can do to obtain approval by using approved products as examples.

In addition, Professor Miyagawa from Osaka University will explain post-market efficacy evaluation using real-world data (RWD).

Post-Marketing Efficacy Evaluation Using RWD (Tentative)

Shigeru Miyagawa, MD, PhD
Professer, Cardiovascular surgery, Osaka University Hospital, Osaka University

Background and Explanation of Draft Guidelines for Post-Marketing Evaluation after Time-Limited Approval (Tentative)

Yoji Sato, PhD
Head, Division of Drugs, National Institute of Health Sciences

Panel Discussion

All Session Speakers and

Shinichi Noda, PhD
Deputy Review Director, Office of Cellular and Tissue-based Products, Pharmaceuticals and Medical Devices Agency (PMDA)

Mitsuaki Chujo, PhD
Phaemacovigicance Department, AnGes Inc.

Yoshihiro Yoshikawa
Regenerative Medicine Reserchcenter, NIPRO Corporation

15:50-16:10 COFFEE BREAK

16:10-16:40 EDUCATIONAL LECTURE 2

SESSION CHAIR

Naoto Watanabe
Asahi Kasei Medical Co., Ltd.

Cell therapy products are often subjected only to minimal purification prior to formulation, and thus the removal or inactivation of potential viral contaminants is often not available. Therefore, in the case of cell therapy products, thorough qualification control of raw materials should be required to reduce virus contamination risks. It is known that cell culture media is one of the typical virus contamination routes. Among biopharmaceutical manufacturers, it becomes gradually recognized that application of virus clearance for cell culture media can provide effective reduction of virus contamination risks, and this can be a workable approach for cell therapy products as well.

In this educational lecture, the lecturer introduces his recent study on applicability of the virus clearance process for a human platelet lysate-contained culture media, which is often used for mesenchymal stem cell propagation. The lecture may lead us to consider more robust virus safety control strategy.

Safeguarding the Virus Safety of Xeno-Free Culture Media for Human Cell Propagation

Thierry Burnouf, PhD
Vice-Dean, distinguished Professor, College of Biomedical Engineering, Taipei Medical University

16:40-17:00 CLOSING REMARKS

Yasuko Terao, PhD
Program VICE-Chair

Takeda Pharmaceutical Co., Ltd.

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Expected contributors:

• Individuals involved in the development of regenerative medical products in pharmaceutical or medical device companies and related industries.
• Individuals involved in the development of regenerative medical products in venture companies or academic institutions.
• Individuals involved in regulatory submissions work for regenerative medical products.

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