APACRM
Asia Partnership Conference of Regenerative Medicine

**Brief Overview**

**Date of the Meeting:**

## Thursday, April 25th, 2024

**Venue (or Zoom):**

## TOKYO MIDTOWN YAESU CONFERENCE

(Please note the change in venue)
4F Main Conference Room
2-2-1 Yaesu, Chuo-ku, Tokyo 104-0028
https://www.yaesu.tokyo-midtown.com/en/conference

**Past Participants:**
**Presenter/Discussant**
Industrial Association: China, India, Japan, Korea, Singapore, Taiwan
Regulatory Authority and Agency: India, Japan, Korea, Singapore, Taiwan
**Observer**
Regulatory Authority: Indonesia, Malaysia, Myanmar, Philippines, Thailand, Vietnam

**One of the key sessions at the APACRM is the discussion featuring panelists invited from regulatory authorities and agencies, as well as facilitators who are APACRM Working Group (WG) members. We would greatly appreciate it if you could register for this conference via the QR code or the link provided below.**

**Registration Form:**

[English site] [Japanese site]

# TOPICS

### Update on Regenerative Medicine from each Participating Industrial Association

We'll share the latest updates on regulations and trends in regenerative medicine across Asia.

### Points to Consider for Cellular Therapy CMC toward New Drug Application (NDA)

This section will outline considerations for manufacturing cellular and tissue-based products, drawing from extensive commercial manufacturing experience.

### Considerations for Conducting Clinical Trials of Regenerative Medicinal Products

We aim to identify key issues related to clinical trials of cellular products within regenerative medicine, consolidating cases and concepts from Asian countries/regions for discussion at the next APACRM.

### Considerations for Preparing an NDA for Gene Therapies

Building upon last year's efforts to identify necessary data for issuing an Investigational New Drug (IND) for Adeno-Associated Virus (AAV), this year we will focus on delineating the data requirements for preparing NDAs for gene therapies, including CAR-T and AAV. We will also explore differing perspectives among countries and regions, aiming to engage in discussions with regulatory authorities and industry stakeholders.

### Non-clinical data package for AAV-based Gene Therapies

Given the recent advancements in the clinical development of AAV, we seek to outline the necessary non-clinical data package required for submitting an IND. This year, we will begin by elucidating Japan's perspective, facilitating future discussions with other countries and regions.

### Compilation of Case Studies on Cell and Gene Technology provided to the Patients as Medical Care or Medical Practice without Market Authorization

With 10 years having passed since the implementation of the Act of Safety Regenerative Medicine in Japan, and revisions scheduled for this year, we will provide insights into the current situation and showcase examples of how unapproved regenerative medicine is being provided in Asian countries such as Taiwan.

# Agenda for APACRM 2024 (7th APACRM)

**Brief Overview of APACRM 2024**
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| Time (JST) | Duration | Session | Chair/Speaker/Panelist |
|---|---|---|---|
| 13:00-13:03 | 3 min | Opening remarks | Yoshitsugu Shitaka, FIRM |
| 13:03-13:10 | 7 min | Introduction | Masayuki (Max) Nomura, FIRM |
| 13:10-14:10 | **Update on Regenerative Medicine from each Participating Industrial Association** | | |
| | 60 min | 10-min presentations from each country/region (Pre-recorded)<br>* on site presentation | Takayuki Nakano, FIRM (Japan) |
| | | | Eugene, J. WANG, CMBA (China) |
| | | | Bryan Choi, CARM & RMAF (Korea) |
| | | | Pawan Kumar Gupta, ABLE (India) |
| | | | Chia-Ning Shen, BPIPO (Taiwan) |
| | | | Srinivasan N Kellathur, SAPI (Singapore)* |
| 14:10-14:40 | **Educational Lecture** | | |
| | 30 min | Points to Consider for Cellular Therapy CMC toward New Drug Application "NDA" | Yukio Mori, FIRM/J-TEC |
| 14:40-15:10 | **Topics for future activities** | | |
| | 30 min | Considerations for Conducting Clinical Trials of Regenerative Medicinal Products | Shigeaki Hayashi, FIRM/J-TEC |
| 15:10-15:20 | 10 min | Break | |
| 15:20-16:40 (80 min) | **Points to Consider for Regulations on Marketing Authorization Application (MAA) for AAV-based Gene Therapies and CAR-T Products** | | |
| | 10 min | Background and Objectives | Masaaki Miyano, FIRM/Chugai |
| | 35 min | Panel Discussion 1:<br>**Regulatory review system**<br>• Requirement of clinical data<br>• ERA review at BLA/MAA<br>• Alliance/reference country | **Panelists:**<br>6 country/region regulators: TBD<br>Alex J. ZHANG, CMBA/Help Tx<br>Srinivasan N Kellathur, SAPI/Roche<br>**Moderator:**<br>Hirokuni Mizoguchi, FIRM/Astellas<br>Ruriko Shinozaki, FIRM/Astellas |
| | 35 min | Panel Discussion 2:<br>**CMC/GMP topics**<br>• Out of specification<br>• In-country testing | **Panelists:**<br>Same with panel discussion 1<br>**Moderator:**<br>Masaki Fujii, FIRM/Mitsui-soko<br>Masaaki Miyano, FIRM/Chugai |
| 16:40-17:10 | **Non-clinical data package for AAV-based Gene Therapies** | | |
| | 30 min | Presentation and Discussion | Yusuke Kagawa, FIRM/Novartis<br>Aki Kito, FIRM/Chugai<br>ALL |
| 17:10-17:55 | **Compilation of Case Studies on Cell and Gene Technology provided to the Patients as Medical Care or Medical Practice without Market Authorization** | | |
| | 45 min | Presentation and Discussion | Kunihiko Suzuki, FIRM/MEDINET<br>James Chieh-liang Lin, MEDIGEN<br>ALL |
| 17:55-18:00 | 5 min | Closing | Ken-ichiro Hata, FIRM |
| 18:30-20:00 | <Reception> Invite all in-person attendants | | |

*Abbreviations:* ABLE, Association of Biotechnology Led Enterprises in India; BPIPO, Biotechnology and Pharmaceutical Industries Promotion Office in Taiwan; CARM, Council for Advanced Regenerative Medicine in Korea; CMBA, China Medical Biotech Association in China; RMAF, Regenerative Medicine Acceleration Foundation in Korea; SAPI, Singapore Association of Pharmaceuticals Industries in Singapore.