International Regulatory Forum of Human Cell Therapy and Gene Therapy Products

- 再生医療等製品国際フォーラム -

PROGRAM

March 16, 2016

Osaka International Convention Center
Osaka, JAPAN

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Organized by

Pharmaceuticals and Medical Devices Agency (PMDA)
The Japanese Society for Regenerative Medicine (JSRM)

With the support from

National Institute of Biomedical Innovation, Health and Nutrition (NIBIOHN)

Under the auspices of

Ministry of Health Labour and Welfare (MHLW)
Forum for Innovative Regenerative Medicine (FIRM)
Japan Pharmaceutical Manufacturers Association (JPMA)

Welcome Message

The purpose of the symposium

This meeting will bring together an outstanding and diverse group of speakers from regulatory agencies, industry, and academia, all of whom are at the forefront of the cell therapy and gene therapy field.
We will identify regulatory points/issues to consider for specific type of products, as well as very critical points/issues for various type of products, which have to be resolved, improved, and/or developed in terms of sound scientific regulation in order to facilitate the availability of products in a rational and timely manner, and which will be valuable globally to public health.

Finally, from global point of view, we will discuss specific issues that are to be scientifically aligned internationally among the regulators, while each regulator has flexibility to decide in a case by case basis, on a risk based approach. We will explore regulatory dialogues and discussions for the future to pursue the data package under minimum consensus for global development of hCTPs.

Organizing Committee

Takao Hayakawa, Kindai Univ., Japan (Chair)
Daisuke Maeda, PMDA, Japan
Yoshiaki Maruyama, PMDA, Japan
Noriyuki Matsumoto, Japan Pharmaceutical Manufacturers Association (JPMA) , Japan
Akifumi Matsuyama, NIBIOHN, Japan
Hajime Miyamoto, FIRM, Japan
Kohji Nishida, The Japanese Society for Regenerative Medicine (JSRM) , Japan
Yoshinori Oie, The Japanese Society for Regenerative Medicine (JSRM) , Japan
Norihisa Sakamoto, PMDA, Japan
Daisaku Sato, PMDA, Japan
Yoji Sato, National Institute of Health Sciences, Japan
Masayuki Shibasaki, FIRM, Japan
Kazuhiro Takekita, PMDA, Japan
Yuzo Toda, FIRM Chairperson, Japan
Kazuhisa Uchida, Japan Pharmaceutical Manufacturers Association (JPMA) , Japan
Akihiro Umezawa, NICHD, Japan
Masayuki Yamato, Tokyo Women's Medical Univ., Japan

Table of Contents

General Information

Location:

Osaka International Convention Center, Osaka, Japan

5-3-51 Nakanoshima, Kita-ku, Osaka City, 530-0005 Japan

TEL : +81-6-4803-5555 (Main)

FAX : +81-6-4803-5620

http://www.gco.co.jp/en/

Osaka International Convention Center

No Smoking:

Smoking is prohibited in all areas of the venue.

No Photos, No Audio Recording:

Photos and audio recording are prohibited.

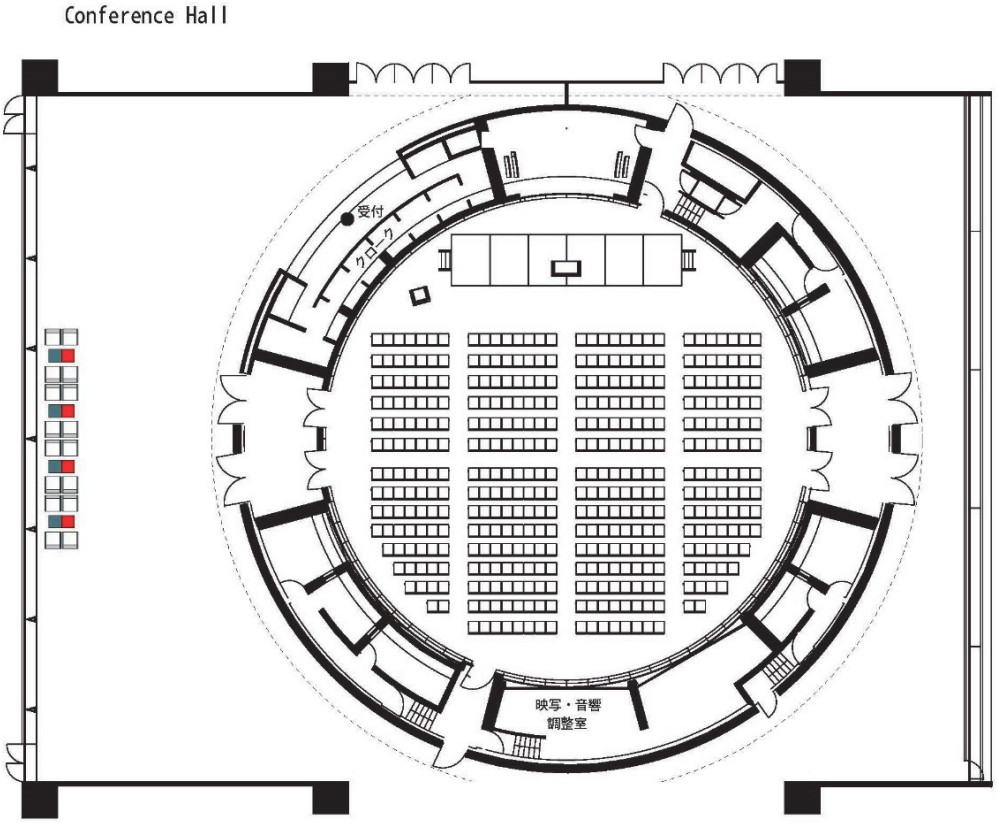
Cellular Phones:

Using cellular phones during the sessions is prohibited.

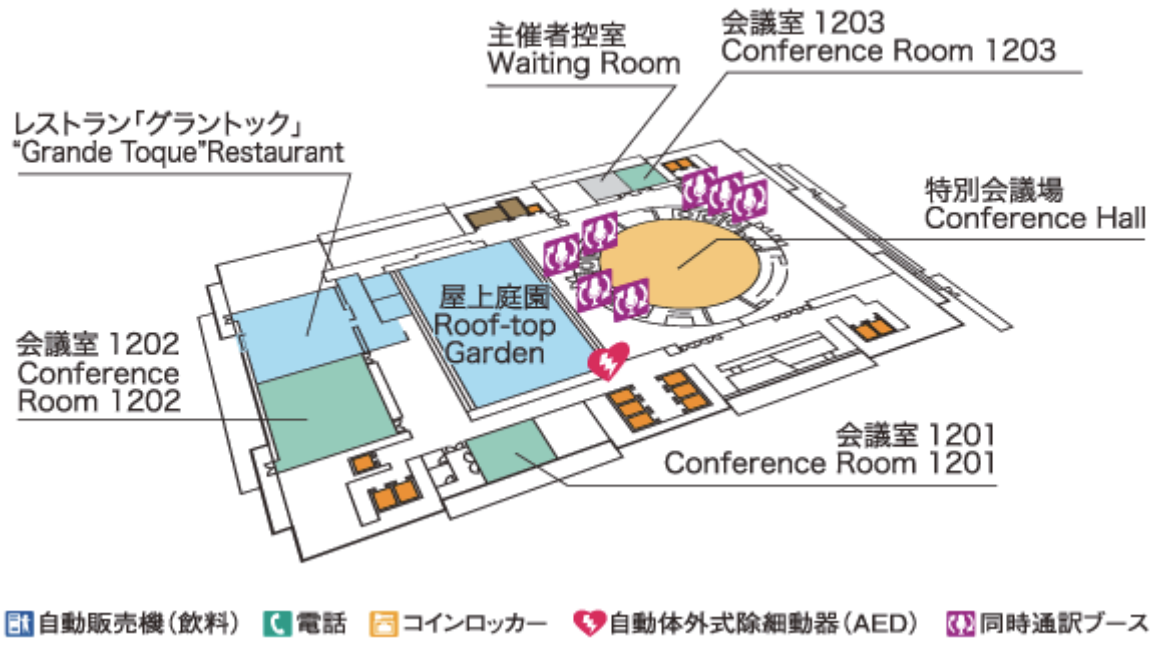
Cellular phones must be turned off or set to silent mode during the sessions.

Conference Venue:

Presentation Hall: Conference Hall



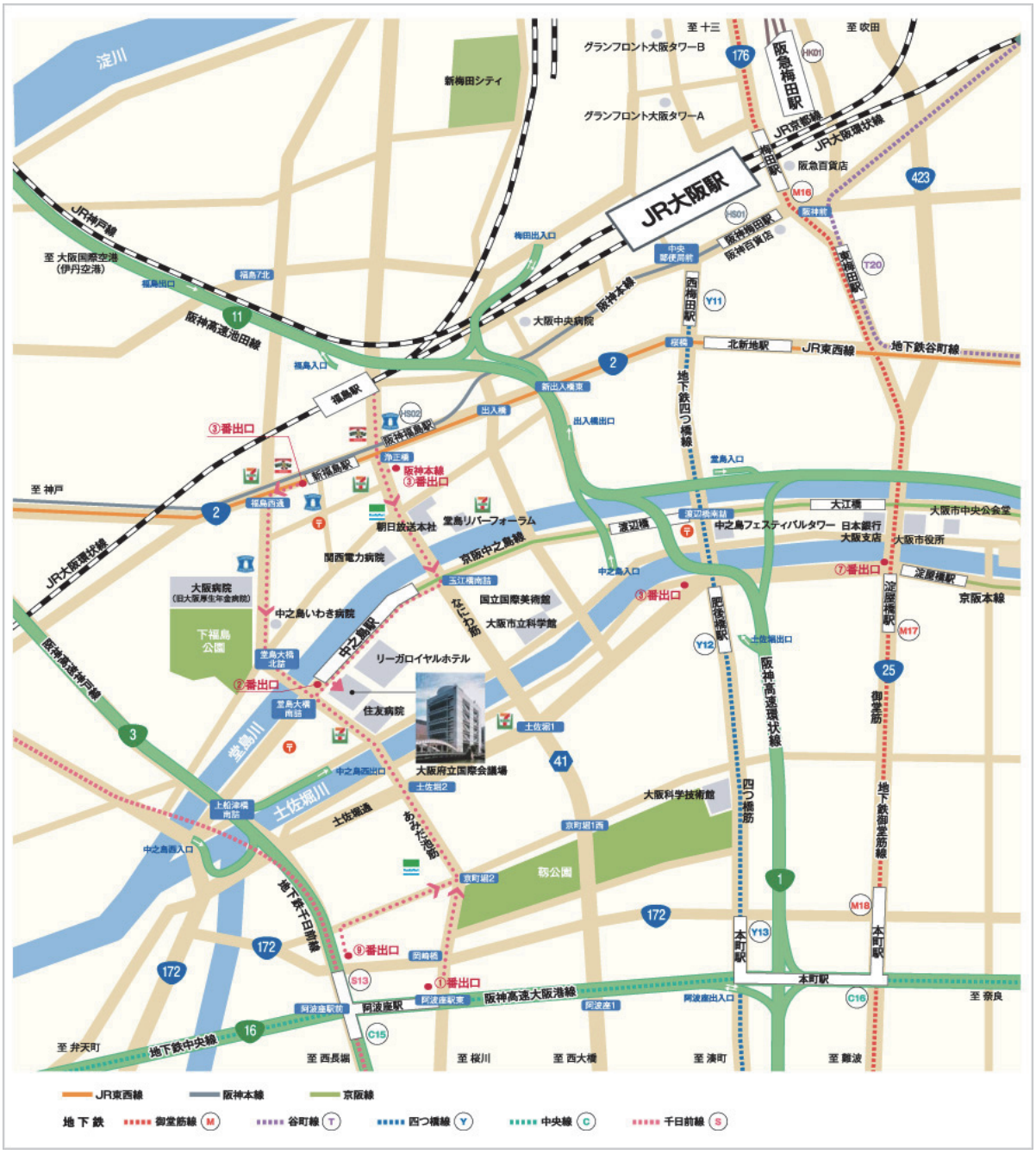
12th Floor



自動販売機（飲料） 電話 コインロッカー 自動体外式除細動器（AED） 同時通訳ブース

Access Information:

In Osaka City, Osaka City Transportation Route Map



Program Schedule

March 16, 2016

8:30 **Registration Open**

9:20 **OPENING**
Opening & Introduction by Chair of Congress JSRM, Japan (Co-sponsor), ***Kohji Nishida***

9:30 Welcome Note by Executive Director, NIBIOHN, Japan (Co-sponsor), ***Suminori Kono***

9:40 Introductory Remarks by Councilor for Pharmaceutical Safety, MHLW, Japan, ***Kazuhiko Mori***

9:50 **Session 1 Keynote Lecture**
Chairs: ***Yuzo Toda*** *(FIRM, Japan)*
Introduction by Chair

9:55 FDA's perspective on cellular and gene therapy regulation
Steven S. Oh (Chief, Cell Therapy Branch, OCTGT, CBER, FDA, USA) ***(teleconference)***

10:20 European perspective on ATMPs
Paula Salmikangas (Chair, CAT/EMA, EU)

10:45 **Break**

11:00 **Session 2 Safety evaluation approaches for hCTPs in reference to ICH S6 and S7 guidelines**
Chairs:, ***Paula Salmikangas*** *(CAT/EMA, EU)* and ***Yoji Sato*** *(NIHS, Japan)*
Introduction by Chair

11:05 2.1 Process related impurity safety evaluation
Takuya Nishimura (PMDA, Japan)

11:25 2.2 Pre-clinical safety evaluation
James McBlane (MHRA, UK)

11:45 2.3 Tumorigenicity
Shin Kawamata (Institute of Biomedical Research and Innovation (IBRI), Japan)

12:05 2.4 Pre-clinical proof of concept and other pre-clinical issues
Ian Harris (Janssen Research & Development, USA)

12:25 **2.5 Panel Discussion**

12:45 **Lunch**

13:45 **Session 3 Quality evaluation approaches for hCTPs in reference to ICH Q5 and Q6 guidelines**
Chairs: ***Junichi Koga*** *(JPMA , Japan) and* ***Takao Hayakawa*** *(Kindai Univ., Japan)*
Introduction by Chair

13:50 3.1 Potency
Anthony Ridgway (Health Canada, Canada)

14:10 3.2 Raw materials and microbiological control
Stephen Wicks (EDQM, EU)

14:30 3.3 Specification and process validation
Yoshiaki Maruyama (PMDA, Japan)

14:50 3.4 Vector characterization of genetically-modified cell therapies
Matthias Renner (PEI, Germany)

15:10 **3.5 Panel Discussion**

15:30 **Break**

15:45 **Session 4 Efficacy evaluation approaches for hCTPs**
Chairs: ***Norihisa Sakamoto*** *(PMDA, Japan) and* ***Akihiro Umezawa*** *(NICHD, Japan)*
Introduction by Chair

15:50 4.1 Study design
Nobuo Kanai (Tokyo Women's Medical Univ. Japan)

16:10 4.2 Clinical issues
Graziella Pellegrini (Università degli Studi di Modena e Reggio Emilia, Italy)

16:30 4.3 Clinical and regulatory issues in the US
Michael Werner (ARM, USA)

16:50 **4.4 Panel Discussion**

17:10 **Session 5 International Development and Regulatory Issues**
Chairs:, ***Anthony Ridgway*** *(Health Canada, Canada) and* ***Daisaku Sato*** *(PMDA, Japan)*
Introduction by Chair

17:15 5.1 Endeavour to minimum consensus package
Takao Hayakawa (Kindai Univ., Japan)

17:45 **5.2 Regulatory Panel**
Moderator: ***Takao Hayakawa*** *(Kindai Univ., Japan) and* ***Anthony Ridgway*** *(Health Canada, Canada)*

Invited Asian regulators (incl. 10min. presentations, updates of Asian regulatory evaluation)
Jeewon Joung (MFDS, Korea)
Srinivasan Kellathur (HSA, Singapore)
Yi-Chu Lin (TFDA, Taiwan)
Daisaku Sato (PMDA, Japan)

James McBlane (MHRA, UK)
Matthias Renner (PEI, Germany)
Paula Salmikangas (CAT/EMA, EU)
Stephen Wicks (EDQM, EU)

19:05 **Session 6 Summary**
Daisaku Sato (PMDA, Japan)

19:15 **CLOSING**
Closing Remark by Chief Executive, PMDA, Japan (Co-sponsor), ***Tatsuya Kondo***

19:20 **CLOSE OF MEETING**

19:30 **Networking Reception**