



5th IABS Cell Therapy Conference: Toward International Convergence of Core Scientific Elements and Evaluation of Human Cell Therapy Products

Organized by
The International Alliance for Biological Standardization
IABS
Tokyo, Japan
February 4-5, 2020

Agenda – February 1, 2020

TUESDAY FEBRUARY 4TH

- 08:30 Registration Open
- 9:15 Photo session
- 9:30 Welcome by Conference Chair, Dr. Yoji SATO (NIHS)
- 09:35 Welcome by Conference Organizer, Prof. Jean-Hugues TROUVIN, International Alliance for Biological Standardization (IABS), France
- 09:45 Keynote address 1
Chair: **Prof. Jean-Hugues Trouvin** (IABS)
K.1: Frontier regenerative medicine in cardiovascular area and overview of JSRM
Dr. Yoshiki Sawa (Osaka Univ. & JSRM)
- 10:25 Keynote address 2
Chair: **Dr. Pierrette Zorzi** (PZorzi-Bioreg, formerly French Drug Agency)
K.2: Points to consider for the evaluation of human cell therapy products
Dr. Takao Hayakawa (Osaka Univ. & IABS)
- 11:05 Session 1 (1) Global regulatory landscape of cell therapy products
Chair: **Dr. Daisaku Sato** (PMDA)
Chair: **Dr. Ivana Knezevic** (WHO)
1.1 Facilitating expedited development of advanced therapy products in the United States
Dr. Steven Oh (CBER/FDA)
1.2 Regulatory perspective on cell therapy development in Europe
Dr. Marcos Timón (AEMPS)
1.3 Regulatory perspectives on cell therapy development in Japan
Dr. Daisaku Sato (PMDA)
- 12:05 Lunch

- 13:30 Session 1 (2)**
- 1.4 Regulatory perspective on cell therapy development in India
Dr. V.G. Somani (CDSCO)
 - 1.5 Regulatory perspective on cell therapy development in Taiwan
Dr. Wen-Yi Hung (Taiwan FDA)
 - 1.6 Regulatory prospective on cell therapy development in Korea
Dr. Jounghee Baek (NIFDS/MFDS)
 - 1.7 Perspectives of the World Health Organization on the development of cell and gene therapy products
Dr. Ivana Knezevic (WHO)
- 14:50 Coffee Break**
- 15:10 Session 2** Sustainable availability of qualified starting materials and raw materials for manufacturing of hCTPs
Chair: **Dr. Takashi Aoi** (Kobe Univ.)
Chair: **Dr. Glyn Stacey** (IABS)
- 2.1 Quality and safety of raw materials (except starting cells)
Dr. Toshimitsu Tanaka (Astellas Pharma Inc.)
 - 2.2 Evaluation and quality control of tissue derived somatic cells as starting materials of hCTPs (including Donor screening, Autologous vs Allogeneic)
Dr. Glyn Stacey (IABS)
 - 2.3 Evaluation and quality control of PSCs as starting materials of hCTPs
Dr. Takashi Aoi (Kobe Univ.)
 - 2.4 From Emily Whitehead to Today: Cell Manufacturing in its Infancy
Dr. Christian Barkey (Barkey GmbH & Co. KG)
- 16:30 Short Break**
- 16:40 Educational Lectures** New analytical technologies for cell therapy products and related materials
Chair: **Dr. Shin Kawamata** (FBRI at KOBE)
- E.1 Analytical technologies for bulk and single cell genomics
Dr. Piero Carninci (RIKEN Center for Integrative Medical Sciences)
 - E.2 Multicomponent analysis of culture supernatant using LC-MS/MS and future application to in-process monitoring of cell manufacturing
Dr. Takashi Suzuki (Shimadzu Corporation)
- 17:40 Short Break**
- 17:45 Panel Discussions** Quality standards of starting materials and biological raw materials for manufacturing of cell therapy product
Moderator: **Dr. Glyn Stacey** (IABS)
Panelists: **Chairs & Speakers of Session 2 & Educational Lectures**
Dr. Yoshiaki Maruyama (PMDA)
- 18:15 Networking Reception**
- 19:45 End of Day 1**

WEDNESDAY FEBRUARY 5TH

- 08:30 Registration Open
- 09:30 **Keynote address 3**
Chair: **Dr. Ken-ichiro Hata** (FIRM)
K.3 The world's first clinical study for corneal cell sheets made from human iPS cells
Dr. Kohji Nishida (Osaka Univ.)
- 10:10 **Session 3** Tumorigenicity Assessment of cell therapy products
Chair: **Dr. Hiroto Bando** (FUJIFILM Corporation)
Chair: **Dr. Lucilia Mouries** (HESI)
3.1 In vivo testing for detection of residual PSCs
Dr. Naoko Tanaka (TERUMO Corporation)
3.2 In vitro testing for detection of residual PSCs
Dr. Akihiko Azuma (FUJIFILM Corporation)
3.3 In vitro testing for detection of transformed cells in cell therapy products
Dr. Kiyoko Bando (Sumitomo Dainippon Pharma Co., Ltd.)
3.4 Non-clinical biodistribution study
Dr. Yoshiteru Kamiyama (Astellas Pharma Inc.)
3.5 HESI CT-TRACS' Tumorigenicity International Experimental Consortium:
a Collaborative Effort to Address Collective Challenges & Needs
Dr. Lucilia Pereira Mouries (HESI)
- 11:25 Short Break
- 11:30 **Panel Discussions** Tumorigenicity Assessment of cell therapy products
Moderator: **Dr. Yoji Sato** (NIHS)
Panelists: **Chairs & Speakers of Session 3**
- 12:00 Lunch Break
- 13:30 **Keynote Address 4**
Chair: **Dr. Akihiro Umezawa** (NCCHD)
K.4.1 The CAR-T Cell Story from European regulator's viewpoint
Dr. Matthias Renner (PEI)
K.4.2 PMDA update: Development of CAR/TCR T Cell Therapy in Japan
Dr. Yoshiaki Maruyama (PMDA)
- 14:30 **Session 4** Characterization and QC of hCTPs and/or their critical intermediates: identification of critical quality attributes (CQA)
Chair: **Dr. Masayuki Yamato** (Tokyo Women's Medical Univ.)
Chair: **Dr. Ingrid Markovic** (Genentec)
4.1 How to identify CQA of the cell product(s) at critical step(s) of mfg. process to ensure consistency of products
Dr. Shin Kawamata (FBRI RDC)
4.2 Developing robust control strategy, identifying CQAs and setting specifications for Cell & Gene Therapy Products
Dr. Ingrid Markovic (Genentec)
4.3 How to establish potency of hCTPs
Dr. Andrew C. Chang & Dr Lisbeth Palm (Novo Nordisk, Inc.)

4.4 Potential comparability study using QA at early development stage : When, How?
Dr. Pierrette Zorzi (PZorzi-Bioreg, formerly French Drug Agency)

15:50 **Coffee Break**

16:10 **Panel Discussions** Characterization, specifications (especially, potency), and comparability of hCTPs
Moderator: **Dr. Ingrid Markovik** (Genentech)
Panelists: **Dr. Masayuki Yamato** (Tokyo Women's Medical Univ.) & **Speakers of Session 4**

16:40 **Short Break**

16:50 **Session 5: Panel Discussions** Toward international convergence of core scientific elements and evaluation of hCTPs

- Potential scientific elements commonly useful for evaluation of most of hCTPs with respect to CMC
- Ongoing discussion/consideration issues
- Subjects to be taken flexible case-by-case approach

Moderator: **Prof. Jean-Hugues Trouvin** (IABS)
Panelists: **Chairs of all the sessions**

17:05 **Wrap Up** Summary of Days 1 & 2, Dr. Yoji Sato (NIHS)

17:25 **Closing Remarks**, **Prof. Jean-Hugues Trouvin** (IABS)

17:35 **End of Day 2**
Close of Conference