4th Cell Therapy Conference:
Manufacturing and Testing of Pluripotent Stem Cells

Organized by
The International Alliance for Biological Standardization
IABS

With Support from and in Collaboration with the
California Institute for Regenerative Medicine
CIRM

Los Angeles, California
June 5 - 6, 2018

Agenda

Scientific Committee

Dr. Abla Creasey	California Institute for Regenerative Medicine (CIRM)
Dr. Stephen Lin	California Institute for Regenerative Medicine (CIRM)
Dr Anthony Lubiniecki	International Alliance for Biological Standardization (IABS), U.S.A.
Professor Glyn Stacey	International Stem Cell Banking Initiative, United Kingdom
Dr. Ivana Knezevic	World Health Organisation (WHO), Switzerland
Dr. Yoji Sato	National Institute of Health Sciences, Japan
Professor Jean-Hugues Trouvin	Paris Descartes University, France
Dr. Elwyn Griffiths	International Alliance for Biological Standardization (IABS) United Kingdom
Dr. Kathy Zoon	Emeritus, National Institutes of Health, U.S.A.
Dr. Robert Deans	BlueRock Therapeutics, U.S.A.
Dr. Kathy Francissen	Genentech, U.S.A.
Dr. Thorsten Gorba	IQVIA, U.S.A.
Dr Karin Hoogendoorn	Leiden University Medical Center, The Netherlands
Professor Krishnendu Roy	Marcus Center for Cell-Therapy, Georgia Institute of Technology, U.S.A.

Day 1 – Tuesday, June 5, 2018

8:00 am **Registration & Welcome Coffee**

8:45 am **Welcome**
Dr. Anthony Lubiniecki, International Alliance for Biological Standardization (IABS), U.S.A.
Dr. Abla Creasey, California Institute for Regenerative Medicine (CIRM) San Francisco, California, U.S.A.

Session 1 – Learning from the current pluripotent space and the development of international standards

Case studies in the challenges of pluripotent stem cell development
Chairs: **Dr. Robert Deans,** BlueRock Therapeutics, Cambridge, Massachusetts, U.S.A.
Dr. Thorsten Gorba, IQVIA, U.S.A

9:00 am Experiences in taking human embryonic stem cells to clinic
Dr. Edward Wirth, Asterias Biotherapeutics, Fremont, California, U.S.A.

9:20 am Delivering differentiated induced pluripotent stem cells for treatment of GvHD
TBD

9:40 am Development of autologous iPSCs to treat blindness
Dr. Kapil Bharti, National Institutes of Health (NIH) / NEI, Bethesda, Maryland, U.S.A.

10:00 am **Panel discussion**
Dr. Robert Deans, Blue Rock Therapeutics, Cambridge, Massachusetts, U.S.A.
Dr. Thorsten Gorba, IQVIA, U.S.A.
Dr. Edward Wirth, Asterias Biotherapeutics, Fremont, California, U.S.A.
Dr. Kapil Bharti, National Institutes of Health / NEI, Bethesda, Maryland, U.S.A.
Dr. Shin Kawamata, Kobe Medical Center / FBRI, Japan

10:45 am **Coffee break**

Learning from international standardisation
Chair: **Dr. Kathryn Zoon,** Emeritus, National Institutes of Health, U.S.A.

11:05 am Historical perspective: the importance of early regulatory guidance for biotherapeutics
Dr. Elwyn Griffiths, International Alliance for Biological Standardization (IABS), United Kingdom

11:25 am WHO standards for cell therapies: key issues in defining quality, safety and efficacy
Dr. Ivana Knezevic, World Health Organization, Geneva, Switzerland

11:45 am **Panel discussion – International regulatory landscape**
Dr. Kathy Zoon, Emeritus, National Institutes of Health, U.S.A.
Dr. Elwyn Griffiths, International Alliance for Biological Standardization (IABS), United Kingdom
Professor Bao Zhu Yuan, National Institutes for Food and Drug Control, Beijing, China
Dr. Mohammad Heidaran, U.S. Food & Drug Administration (FDA), Silver Spring, Maryland, U.S.A.
TBC, Ministry of Food and Drug Safety (MFDS), Republic of Korea

12:30 pm **Lunch**

Session II - Bioanalytics and comparability (non-clinical and quality control)

Chair: **Dr. Kathy Francissen,** Genentech**,** South San Francisco, U.S.A.

1:30 pm Cellular product manufacturing and comparability considerations
Dr. Mohammad Heidaran, U.S. Food & Drug Administration (FDA), Silver Spring, Maryland, U.S.A.

1:50 pm Experiences in establishing comparability of cell derived vaccines
TBC

2:10 pm Characterisation of CART cells
Dr. Shirley Bartido, Cellectis, New York, U.S.A.

2:30 pm Bioanalysis and delivery of immune-modulatory cell therapies
Dr. Stuart Abbot, Fate Therapeutics, La Jolla, California, U.S.A.

2:50 pm Large scale haplobanking and comparability issues
Dr. Stephen Sullivan, GAiT, United Kingdom

3:10 pm The Universal Cells approach to avoiding immune rejection
Dr. Hardy T. S. Kagimoto, Healios K.K, Tokyo, Japan

3:40 pm **Coffee break**

4:10 pm **TBC**
Professor Jack Price, National Institute for Biological Standards and Control-MHRA, Hertfordshire, United Kingdom

4:50 pm Developing predictive assays for qualification of hESC-derived dopaminergic neurons for treatment of Parkinson's Disease
Dr. Agnete Kirkeby, University of Lund, Sweden

5:10 pm **EMA perspectives on comparability**
European Medicines Agency, London, United Kingdom / Amsterdam, The Netherlands

5:30 pm **Panel discussion - Assay development focusing on characterisation, potency assays and comparability**
Professor Krishnendu Roy, Marcus Center for Cell-Therapy, Georgia Institute of Technology, Atlanta, Georgia, U.S.A
Dr. Kathy Francissen, Genentech**,** South San Francisco, U.S.A.
Dr. Mohammad Heidaran, U.S. Food & Drug Administration (FDA), Silver Spring, Maryland, U.S.A.
Dr. Shirley Bartido, Cellectis, New York, New York, U.S.A.
Dr. Stuart Abbot, Fate Therapeutics, La Jolla, California, U.S.A.
Dr. Stephen Sullivan, GAiT, United Kingdom
Dr. Hardy T.S. Kagimoto, Healios K.K., Tokyo, Japan
Professor Jack Price, National Institute for Biological Standards and Control-MHRA, Hertfordshire, U.K.
Dr. Agnete Kirkeby, University of Lund, Sweden
TBC, European Medicines Agency, London, United Kingdom / Amsterdam, The Netherlands

Provisional topics for discussion:

- What is different about cellular therapies which could affect their control and standardisation?
- What measures could be taken to identify key quality attributes of cell therapy products which could be used to demonstrate comparability?
- Explore definitions of potency, purity and identity for cellular therapies
- What should a potency assay be seeking to demonstrate?
- Do we have good examples of potency assays?

6:00 pm **End of Day 1**

Day 2 – Wednesday, June 6, 2018

Session III – Tumorigenicity Testing

***In vivo* and *in vitro* methods, including genetic changes: correlations, issues, the way forward**
Chairs: **Mercedes Serabian**, U.S. Food & Drug Administration (FDA), Silver Spring, Maryland, U.S.A.
Dr. Yoji Sato, National Institute of Health Sciences, Kawasaki, Japan

8:30 am Tumorigenicity testing: a regulatory perspective
Mercedes Serabian, U.S. Food & Drug Administration (FDA), Silver Spring, Maryland, U.S.A.

8:50 am **Creating efficient tumorigenicity assays**
Dr. Shawna Jackman, Charles River Laboratories, Horsham, Pennsylvania; U.S.A.

9:10 am Imaging technology and biodistribution studies
Professor Kevin Park, University of Liverpool, United Kingdom

9:30 am Genetic and epigenetic stability of human pluripotent stem cells
Professor Martin Pera, International Stem Cell Initiative / Jackson Laboratories, Bar Harbor, Maine, U.S.A.

9:50 am **Coffee break**

Preclinical models for cell biodistribution and tumorigenicity

10:20 am Design and validation of ESC removal in the manufacturing process
Dr. Craig Halberstad, Asterias Biotherapeutics, Fremont, California, U.S.A.

10:40 am Design of in vivo tumorigenicity test of iPSC-derived cell product: Kobe experience
FBRI (Foundation for Biomedical Research and Innovation)
Dr. Shin Kawamata, Kobe Medical Center / Foundation for Biomedical Research and Innovation (FBRI), Kobe, Japan

11:00 am Preclinical models for cell biodistribution and tumorgenicity: the CT-TRACS project
Dr. Yoji Sato, National Institute of Health Sciences, Kawasaki, Japan

11:20 am **Panel discussion – Strengths and weaknesses of current standards and discussion of several new methods**
Mercedes Serabian, U.S. Food & Drug Administration (FDA), Silver Spring, Maryland, U.S.A.
Dr. Yoji Sato, National Institute of Health Sciences, Kawasaki, Japan
Dr. Shawna Jackman, Charles River Laboratories, Horsham, Pennsylvania; U.S.A.
Professor Kevin Park, University of Liverpool, United Kingdom
Professor Martin Pera, International Stem Cell Initiative / Jackson Laboratories, Bar Harbor, Maine, U.S.A.
Dr. Craig Halberstad, Asterias Biotherapeutics, Fremont, California, U.S.A.
Dr. Shin Kawamata, Kobe Medical Center / Foundation for Biomedical Research and Innovation (FBRI), Kobe, Japan
Dr. Joy Cavagnaro, Access BIO, Boyce, Virginia
Dr. Jiwen Zang, Alliance for Regenerative Medicine, Standards Coordinating Body, Philadelphia, Pennsylvania, U.S.A.

Provisional topics:

- Relevance of current tumorigenesis tests and selection of animal models
- Utility of animal models for preclinical testing
- Establishing acceptable levels of sensitivity for detection of tumorigenic cells
- What should be tested, source cells, cell banks, post-manufacture intermediates production or product?

12:15 **Lunch**

Session IV – Manufacture, Storage and Shipment

Chairs: **Dr. Karin Hoogendoorn**, Leiden University Medical Center, The Netherlands
Dr. Ivana Knezevic, World Health Organization (WHO), Geneva, Switzerland

1:00 pm Fortuna Fix experiences in automation
Dr. Jan-Eric Ahlfors, Fortuna Fix, Laval, Canada

1:20 pm Aseptic processing and particulates in cell therapies
Dr. Carl Burke, J&J, Horsham, Pennsylvania, U.S.A.

1:40 pm Experiences working with new product developers
Dr. Behnam Ahmadian, Lonza, Basel, Switzerland

2:00 pm Enhancing economic reality for cell based medicines
Dr. Benjamin Le Quéré, Saint Gobain Performance Plastics, Paris, France

2:20 pm Preservation and cold chain strategies for cell therapy
Dr. William Singleton, GE Healthcare, Hertfordshire, United Kingdom

2:40 pm **Coffee break**

3:00 pm **Panel discussion – Manufacturing standards, preservation and shipment**
Dr. Karin Hoogendoorn, Leiden University Medical Center, The Netherlands
Dr. Ivana Knezevic, World Health Organization (WHO), Geneva, Switzerland
Dr. Jan-Eric Ahlfors, Fortuna Fix, Laval, Canada
Dr. Carl Burke, J&J, Horsham, Pennsylvania; U.S.A.
Dr. Thomas Fellner, Lonza, Basel, Switzerland
Dr. Benjamlin Le Quéré, Saint Gobain Performance Plastics, Gaithersburg, Maryland, U.S.A.
Dr. John Morris, GE Healthcare, Hertfordshire, United Kingdom
Dr. Tatsuo Heki, Fujifilm Co., Tokyo, Japan
Dr. Soren Knudsen, Cryoport, Irvine, California, U.S.A.

Provisional topics:

- What are the new challenges for development of automated cell culture systems?
- What are the optimal approaches to implementing automated processes?
- How can ISO standards contribute to improved bioprocessing?
- What approach should be taken to setting expiration dates for cryopreserved products?
- What does the developer need to understand about the preservation process?

Session V – Conclusions

3:45 pm **Summary of key issues and conclusions**
Professor Glyn Stacey, International Stem Cell Banking Initiative, United Kingdom

3:55 pm **Presentation to Dr. John Petricciani,** International Alliance for Biological Standardization (IABS), U.S.A.
Dr. Ivana Knezevic, World Health Organization (WHO), Geneva, Switzerland
Dr. Anthony Lubiniecki, International Alliance for Biological Standardization (IABS), U.S.A.

4:00 pm **End of meeting**