

APA 2020 SHORT COURSE AGENDA

Current Trends and Future Direction in the Development of Cell and Gene Therapies

Course Coordinators:

Cindy Xia, Takeda Nagendra Chemuturi, Takeda Johanna Mora, BMS

MONDAY, OCTOBER 19

1:00-1:40 pm PLENARY SPEAKER

In Vivo Gene Therapy as a New Paradigm in Medicine: The Road to Today and Beyond

Guangping Gao, PhD

Horae Gene Therapy Center & Weibo Li Institute for Rare Diseases Research, University of Massachusetts Medical School

1:40 - 1:45 PM **Q & A** 1:45 - 2:00 PM **BREAK**

2:00-2:30 pm

Development of a Risk based Immunogenicity Strategy for Support of AAV based Gene Therapies in Clinic

Vibha Jawa, PhD

Executive Director, Nonclinical Disposition and Bioanalysis, Bristol Myers Squibb

2:30 - 2:45 PM **0 & A**

2:45-3:15 pm

The Pharmacokinetics of AAV Gene Therapies – Biodistribution and Shedding

Mark Milton, PhD

Novartis Institutes for BioMedical Research

3:15 - 3:30 PM **Q & A**

TUESDAY, OCTOBER 20

1:00-1:40 pm PLENARY SPEAKER

Preclinical Assessment of Cell and Gene Therapy Products to Support an IND

Christopher Saeui, PhD

Cell and Gene Therapy Reviewer at FDA

1:40 - 1:45 PM **Q & A** 1:45 - 2:00 PM **BREAK**

2:00-2:30 pm

An Integrated Approach to Investigating CART Cell Immuno-biology, a Pharmacology Perspective

Piotr L. Pierog, PhD

Head of Cell Product Clinical Development, Cell Therapy Unit, Takeda Pharmaceuticals Inc.

2:30 - 2:45 PM **Q & A**

2:45-3:15 pm

Considerations for the Bioanalysis of CARTs

Timothy Mack, PhD

Senior Principal Scientist at Celgene/BMS

3:15 - 3:30 PM **Q & A**

