



RANS LATING 17-19 November 2022. Boston, USA Pluripotent DISCOVERIES Stem Cells

DRAFT Subject to Change

THURSDAY, 17 NOVEMBER

8:00 AM - 9:00 AM

Innovation Showcase

INTRODUCTION TO THE CELL AND GENE THERAPY CATAPULT

Presented by: Cell and Gene Therapy Catapult

Grand Ballroom Salons 1-3

9:00 AM - 9:25 AM

BREAK

Grand Ballroom Foyer

9:25 AM - 11:20 AM

Welcome and Opening Remarks

PRECLINICAL STRATEGIES

Sponsored by: Astellas Institute for Regenerative Medicine

Chair: Derek Hei, Clade Therapeutics, USA

Grand Ballroom Salons 1-3

9:30 AM - 10:00 AM

Opening Keynote

Douglas A. Melton, Harvard University and Vertex Pharmaceuticals, USA

STEM CELL-DERIVED ISLETS FOR THE TREATMENT OF DIABETES

10:00 AM - 10:30 AM

Su-Chun Zhang, Duke-NUS Medical School, Singapore and University of Wisconsin, USA CELL THERAPY FOR NEUROLOGICAL DISORDERS

10:30 AM - 11:00 AM

April Craft, Boston Children's Hospital, USA

OFF THE SHELF TISSUE FOR CARTILAGE REPAIR

11:00 AM - 11:20 AM

Kareen Coulombe, Brown University, USA

HEART REGENERATION WITH HUMAN ENGINEERED MYOCARDIUM

11:20 AM - 1:00 PM

LUNCH BREAK

Grand Ballroom Salons 4-7

11:45 AM - 12:45 PM

Innovation Showcase

DIFFERENTIATION TO MEGAKARYOCYTES TO SUPPORT TRANSLATIONAL RESEARCH AND SCALABLE EXPANSION OF HUMAN PLURIPOTENT STEM CELLS IN SUSPENSION CULTURE

Presented by: STEMCELL Technologies

Grand Ballroom Salons 1-3

1:00 PM - 2:30 PM

SELECTING YOUR CELL LINE: DERIVATION AND CHARACTERIZATION

Sponsored by: ElevateBio

Chair: Melissa Carpenter, ElevateBio, USA

Grand Ballroom Salons 1-3

1:00 PM - 1:30 PM

Tenneille Ludwig, WiCell Research Institute, USA

TRANSLATIONAL SUCCESS: MINDING YOUR P's AND Q'S

1:30 PM - 2:00 PM

Jennifer Dashnau, Century Therapeutics, USA

REGULATORY CONSIDERATIONS FOR GENETICALLY-ENGINEERED, INDUCED PLURIPOTENT STEM-CELL DERIVED ALLOGENEIC CELL THERAPIES: A PROPOSED RISK-BASED APPROACH FOR ADDRESSING CHEMISTRY, MANUFACTURING, AND CONTROLS

2:00 PM - 2:30 PM

Nissim Benvenisty, Hebrew University, Israel

GENETIC AND EPIGENETIC CHARACTERIZATION OF HUMAN PLURIPOTENT STEM CELLS

2:30 PM - 2:50 PM

REFRESHMENT BREAK Grand Ballroom Salons 4-7

2:50 PM - 4:20 PM

WHAT IS IN YOUR PRODUCT?

Chair: Kapil Bharti, National Institutes of Health, USA

Grand Ballroom Salons 1-3

2:50 PM - 3:20 PM

Deborah Hursh, PhD, US Food and Drug Administration, USA

CMC CONSIDERATIONS FOR PLURIPOTENT STEM CELL DERIVED PRODUCTS: FDA PERSPECTIVES *Virtual Presentation

3:20 PM - 3:50 PM

Bob Valamehr, Fate Therapeutics, Inc, USA

FT819 AND FT596: FIRST-OF-KIND OFF-THE-SHELF CAR19 T-CELL AND NK CELLS FOR B CELL MALIGNANCIES

3:50 PM - 4:20 PM

Angela Keightley, BlueRock Therapeutics, Canada

ANALYTICAL DEVELOPMENT STRATEGIES FOR EARLY-STAGE CELL THERAPY PRODUCT CHARACTERIZATION

4:30 PM - 6:30 PM

WELCOME RECEPTION & POSTER NETWORKING SESSION Grand Ballroom Salons 4-7

4:30 PM - 5:15 PM

POSTER SESSION I ODD

5:45 PM - 6:15 PM

POSTER SESSION II EVEN

FRIDAY, 18 NOVEMBER

8:00 AM - 9:00 AM

Innovation Showcase

NEXT-GENERATION IN-VITRO ASSAYS: CHARACTERIZING THE ACTIVITY OF HUMAN IPSC-DERIVED

NEURONS IN 2D AND 3D CULTURES AT HIGH RESOLUTION

Presented by: MaxWell Biosystems

Grand Ballroom Salons 1-3

*This Innovation Showcase will be available in-person and on the virtual meeting platform.

9:00 AM - 9:30 AM

BREAK

Grand Ballroom Foyer

9:30 AM - 11:20 AM

NEW TECHNOLOGIES/WHERE ARE WE GOING?

Chair: Sonja Schrepfer, Sana Biotechnology and University of California, San Francisco, USA Grand Ballroom Salons 1-3

9:30 AM - 10:00 AM

Kapil Bharti, National Institutes of Health, USA

DEVELOPING AUTOLOGOUS IPS CELL THERAPY FOR MACULAR DEGENERATION: FROM BENCH-TO-BEDSIDE

10:00 AM - 10:30 AM

Peter Zandstra, University of British Columbia, Canada

SIGNALING DYNAMICS DURING T-CELL DEVELOPMENT FROM PLURIPOTENT STEM CELLS *Virtual Presentation

10:30 AM - 11:00 AM

Howard Kim, New York Stem Cell Foundation Research Institute, USA

LEVERAGING AUTOMATION AND AI TO STREAMLINE IPSC PRODUCTION AND RESEARCH

11:00 AM - 11:20 AM

Kévin Alessandri, TreeFrog Therapeutics, France

FROM 50 MILLION TO 15 BILLION HUMAN IPS CELLS WITHIN A WEEK: HIGHLY REPRODUCIBLE EXPONENTIAL IPS EXPANSION IN 10L BIOREACTORS WITH MAINTENANCE OF CELL QUALITY

11:20 AM - 11:40 AM

Kiera Dwyer, Brown University, USA

ONE BILLION CELLS: ENGINEERING CARDIAC TISSUE WITH HUMAN INDUCED PLURIPOTENT STEM CELL DERIVED CARDIOMYOCYTES FOR THERAPEUTIC IMPACT

11:40 AM - 1:00 PM

LUNCH BREAK

Grand Ballroom Salons 4-7

11:50 AM - 12:50 PM

Innovation Showcase

MANUFACTURING THE NEXT GENERATION OF PLURIPOTENT STEM CELL-BASED THERAPIES

Presented by: Pluristyx, Inc.

Grand Ballroom Salons 1-3

*This Innovation Showcase will be available in-person and on the virtual meeting platform.

1:00 PM - 2:30 PM

IMMUNOLOGY FOR CELL THERAPIES

Chair: Bob Valamehr, Fate Therapeutics, Inc, USA

Grand Ballroom Salons 1-3

1:00 PM - 1:30 PM

Ran Jing, Harvard Medical School/Boston Children's Hospital, USA

EPIGENETIC ENGINEERING OF IPSC-DERIVED T CELLS

1:30 PM - 2:00 PM

Sonja Schrepfer, Sana Biotechnology Inc. and University of California, San Francisco, USA ENGINEERING OF ALLOGENEIC DONOR CELLS FOR ACCEPTANCE BY THE HOST'S IMMUNE SYSTEM

2:00 PM - 2:30 PM

Shin Kaneko, Kyoto University, Japan

*Virtual Presentation

2:30 PM - 3:00 PM

REFRESHMENT BREAK

Grand Ballroom Salons 4-7

3:00 PM - 4:30 PM

BALANCING SCIENCE AND REGULATORY REQUIREMENTS IN EMERGING FIELDS

Sponsored by: Cellino

Chair: Jane Lebkowski, Regenerative Patch Technologies, USA

Grand Ballroom Salons 1-3

3:00 PM - 3:30 PM

Gesine Paul-Visse, Lund University and Skånes University Hospital, Sweden

STEM-PD TRIAL: CLINICAL TRIAL DESIGN AND REGULATORY CHALLENGES

*Virtual Presentation

3:30 PM - 4:00 PM

Anthony Oro, Stanford University School of Medicine, USA

SCALEABLE, SAFE, AND EFFECTIVE GENETICALLY-CORRECTED TISSUE MANUFACTURING AND DELIVERY

4:00 PM - 4:30 PM

Melissa Carpenter, ElevateBio, USA

DEVELOPING PLURIPOTENT STEM CELL-DERIVED THERAPIES

4:30 PM - 4:50 PM

BREAK

Grand Ballroom Salons 4-7

4:50 PM - 5:50 PM

CAREER DEVELOPMENT SESSION

Chair: Marinna Madrid, Cellino Biotech, USA

Panelist: Julie Perlin, PhD, Ten Bridge Communications

Panelist: Austin Thiel, PhD, ElevateBio

Panelist: Ibrahim Domian, MD, PhD, BlueRock Therapeutics

Panelist: Kimberly Snyder, MS, STEMCELL Technologies

Grand Ballroom Salons 1-3

SATURDAY, 19 NOVEMBER

8:00 AM - 9:00 AM

Innovation Showcase

CANCER CELL THERAPY AT ASTELLAS

Presented by: Astellas Institute for Regenerative Medicine

Grand Ballroom Salons 1-3

9:00 AM - 9:30 AM

BREAK

Grand Ballroom Foyer

9:30 AM - 11:00 AM

HOW TO ACCELERATE WITH ACCELERATED DESIGNATIONS

Chair: Anthony Oro, Stanford University, USA

Grand Ballroom Salons 1-3

9:30 AM - 10:00 AM

Masayo Takahashi, Vision Care Inc., Japan

RETINAL CELL THERAPY AS A SUSTAINABLE CATEGORIZE MEDICINE

*Virtual Presentation

10:00 PM - 10:30 PM

Jane Lebkowski, Regenerative Patch Technologies, USA

DEVELOPMENT OF MANUFACTURING PROCESSES FOR PLURIPOTENT CELL BASED THERAPIES: CONSIDERATIONS FOR ACCELERATED APPROVAL PATHWAYS

10:30 AM - 11:00 AM

Jacqueline Barry, PhD, Cell and Gene Therapy Catapult, UK

EU AND UK ACCELERATED APPROVAL PATHWAYS

11:00 AM - 12:30 PM

LUNCH BREAK

Grand Ballroom Salons 4-7

11:15 AM - 12:15 PM

Innovation Showcase

PAVING THE ROAD TO TRANSLATION THROUGH SCALABLE, MODULAR, AUTOMATED AND CLOSED

MANUFACTURING

Presented by: ARMI | BioFab, USA

Grand Ballroom Salons 1-3

12:30 PM - 2:00 PM

DELIVERING YOUR CELL PRODUCT: STRATEGIES FOR SUCCESS

Sponsored by: Vertex Pharmaceuticals

Chair: Derek Hei, Clade Therapeutics, USA

Grand Ballroom Salons 1-3

12:30 PM - 1:00 PM

Timothy Kamp, University of Wisconsin, USA

HUMAN IPSC-DERIVED CARDIAC PROGENITOR CELLS FOR MYOCARDIAL REPAIR

1:00 PM - 1:30 PM

Cory R. Nicholas, Neurona Therapeutics, USA

PHASE I/II CLINICAL INVESTIGATION OF HUMAN INHIBITORY NEURON CELL THERAPY FOR CHRONIC FOCAL EPILEPSY

1:30 PM - 2:00 PM

Closing Keynote

Peter Marks, US Food and Drug Administration, USA

THE REGULATORY FRAMEWORK FOR CELLULAR THERAPIES IN THE UNITED STATES