CELL GENE

MEETING ON THE MESA

DAY 1

PARTNERING FORUM

WEDNESDAY | OCTOBER 3, 2018
ESTANCIA LA JOLLA HOTEL & SPA

6:45am – 9:15am | REGISTRATION AND BREAKFAST
Sponsored by SCM Lifescience

7:15am – 8:45am | CONCURRENT WORKSHOPS

DOING BUSINESS IN JAPAN WORKSHOP

Magnolia Room | 7:15am – 8:45am
In Partnership with the Forum for Innovative Regenerative Medicine (FIRM)

The Innovative Regulatory Pathways for Commercialization in Japan – Latest Progress and Outlook

Japan takes pride in its forward-looking regulatory system for regenerative medicine products that include cell and gene therapies, and has further upgraded its scheme by deploying the Sakigake Designation System since 2016. An introduction to the system was presented at last year's Cell & Gene Meeting on the Mesa. This workshop will dive even further into the system this year, and will be augmented with firsthand experiences from three progressive clinical development companies from overseas.

Workshop Facilitator:
Kunihiko Suzuki, Vice Chairman, Forum for Innovative Regenerative Medicine (FIRM); Vice Chairman and Member of the Board, MEDINET Co.

7:15am – 7:20am | Welcome Remarks
Speaker:
Yuzo Toda, Chairman, Forum for Innovative Regenerative Medicine (FIRM)

7:20am – 7:30am | Japan: Best Place for Commercialization of Regenerative Medicine
Speaker:

GENE-BASED MEDICINE DEVELOPMENT WORKSHOP

Learning Theater | 7:15am – 8:45am
Sponsored by Aldevron and MaxCyte

Assessing the challenges and implementing solutions to improve treatments

7:15am – 8:00am | Session 1: Gene Editing: Overcoming the Technical Challenges of CRISPR

Gene editing holds the potential to revolutionize how we approach gene-based medicine and the tools provided by the CRISPR/Cas9 system enable unprecedented opportunities to treat disease. To realize this potential, the safety and efficacy of these methods must be established. This panel will discuss the technical challenges of CRISPR/Cas9 and the work underway to bring these new therapeutic approaches to patients.

Chair:
James Brown, Ph.D., VP, Corporate Development, Aldevron
Speakers:
James Burns, Ph.D., President and CEO, Casebia Therapeutics
Stacy Coen, VP, Business Development, Editas Medicine
Rachel Haurwitz, Ph.D., President and CEO, Caribou Biosciences
Jennifer King, Ph.D., SVP, Business Development, Intellia Therapeutics

SUCCESSFUL STRATEGIES IN THE DESIGN AND DELIVERY OF A CELL/GENE THERAPY CLINICAL DEVELOPMENT PROGRAM WORKSHOP

Ballroom 2 | 7:15am – 8:45am
Sponsored by IQVIA

Streamlined, rigorous and effective clinical trials translate to greater and faster knowledge generation of a therapy's risk-benefit profile. For cell and gene therapies, unique issues impact the design and delivery of a clinical development program including operational and regulatory hurdles.

This session will provide insight on the challenges developers of cell and gene therapies have encountered in executing clinical development programs including: manufacturing/supply chain complexities, access to data, selection of sites and investigators, regulatory and start-up, patient recruitment, competing therapies under development and other critical areas. Strategies that were successful in overcoming those challenges and the implications for strategic clinical development will be discussed. Perspectives from manufacturers and clinical trial sponsors, sites and investigators based in the U.S., EU and APAC will be represented.

7:15am – 7:20am | Welcome Remarks
Speaker:
Caitilin Hamill-Ward, Senior Director, Regulatory Affairs, IQVIA

Masahiro Uemura, Director, Bio-Industry Division, Commerce and Information Policy Bureau, Ministry of Economy, Trade and Industry (METI)

7:30am – 7:40am | Regenerative Medicine Product Regulations in Japan – Enhancing the Development of Advanced Therapy
Speaker:
Kiyohito Nakai, Ph.D., Director, Medical Device Evaluation Division, Pharmaceutical Safety and Environmental Health Bureau, Ministry of Health, Labor and Welfare (MHLW)

7:40am – 7:50am | Experiences from Japan: Sakigake Designation System for Regenerative Medical Products
Speaker:
Yoshiaki Maruyama, Ph.D., Review Director, Office of Cellular and Tissue-based Products, Pharmaceuticals and Medical Devices Agency (PMDA)

7:50am – 8:05am | Development of an "Off the Shelf" Cell Therapy for Ischemic Stroke and Other Indications Under the Regenerative Medicine Regulatory Framework in Japan
Speaker:
Gil Van Bokkelen, Ph.D., Chairman and CEO, Athersys

8:05am – 8:20am | Autologous CD34 Cell Therapy for Critical Limb Ischemia: A Long-term Japanese-American Partnership
Speaker:
Douglas Losordo, M.D., EVP, Global Head of Research and Development and Chief Medical Officer, Caladrius Biosciences

8:20am – 8:35am | Introduction of Gene Therapy for Rare Genetic Diseases in Japan
Speaker:
David Lennon, Ph.D., President, AveXis

8:35am – 8:40am | Q&A

8:40am – 8:45am | Closing Remarks
Speaker:
Yoshitsugu Shitaka, Ph.D., Vice Chairman, Forum for Innovative Regenerative Medicine (FIRM); President, Astellas Institute for Regenerative Medicine (AIRM)

Bill Lundberg, M.D., Senior Advisor, CRISPR Therapeutics

8:00am – 8:45am | Session 2: CMC Solutions for Viral and Non-Viral Vector Gene Therapy

This session will discuss some of the unique hurdles associated with engineered cells including the distinctive challenges associated with both viral and non-viral approaches. Specifically, the panelists will address the impacts of gene delivery approaches on study design – manufacturing, clinical and regulatory considerations.

Chair:
Jessica Carmen, Ph.D., Director of Business Development, Cellular Therapy Partnerships, MaxCyte
Speakers:
Steven Howe, Ph.D., Director, Cell and Gene Therapy Process Research, GSK
Chris Mason, M.D., Ph.D., Chief Science Officer, AVROBIO
Paul McCormac, Ph.D., Category Lead Rare Disease, Biotherapeutic Pharmaceutical Sciences, Pfizer
Kyriacos Mitrophanous, Ph.D., Chief Scientific Officer, Oxford BioMedica

7:20am – 8:00am | Session 1: U.S.-based Clinical Programs
Chair:
Jami Norris, VP of Clinical Project Management – Internal Medicine, IQVIA
Speakers:
Peter Altman, Ph.D., President and CEO, BioCardia
Robert Deans, Ph.D., Chief Technology Officer, BlueRock Therapeutics
Felix Hsu, SVP and Head, Advanced Therapies Unit, WuXi AppTec
Dan Kaufman, M.D., Ph.D., Professor of Medicine, Division of Regenerative Medicine; Director of Cell Therapy, UC San Diego
Edward Wirth, M.D., Ph.D., Chief Medical Officer, Asterias Biotherapeutics

8:00am – 8:40am | Session 2: EU and APAC-based Clinical Programs
Chair:
Adrian McKemey, Ph.D., SVP and Managing Director, Consulting Services; Global Head, Research and Development Strategy Solutions, IQVIA
Speakers:
Blake Anson, Ph.D., Director, Strategic Alliances, Fujifilm Cellular Dynamics
Gisèle Deblandre, Ph.D., Scientific and Project Management Director, MaSTherCell
Hardy Kagimoto, M.D., Chairman and CEO, Healios K.K.
María Pascual, VP Regulatory Affairs and Corporate Quality, TiGenix
Joseph Petroziello, VP, Scientific and Corporate Communications, BrainStorm Cell Therapeutics

8:40am – 8:45am | Closing Remarks
Speaker:
Adrian McKemey, Ph.D., SVP and Managing Director, Consulting Services; Global Head, Research and Development Strategy Solutions, IQVIA

9:00am | GENERAL SESSION AND PARTNERING OPENS

9:00am – 9:15am **WELCOME REMARKS** *{Ballroom 1}*

Speakers:

Janet Lambert, CEO, Alliance for Regenerative Medicine (ARM)
Robert Preti, Ph.D., Chairman, Alliance for Regenerative Medicine (ARM); President and CEO, Hitachi Chemical Advanced Therapeutics Solutions; GM, Hitachi Chemical Regenerative Medicine Business Sector

9:15am – 10:15am **PLENARY SESSION: CHARTING THE PATH – LESSONS FROM THE PIONEERS OF CELL AND GENE THERAPY COMMERCIALIZATION** *{Ballroom 1}*

This session will explore how far we have come in recent years with the approval of the first gene and cell therapy products and a burgeoning industry pipeline expected to produce numerous additional new therapies in the coming years. Executives from three of the companies who have been at the forefront of cell and gene medicine will share their perspectives on the major scientific, regulatory and technical developments of recent years that have contributed most to the sector's current success.

Chair:

Robert Preti, Ph.D., President and CEO, Hitachi Chemical Advanced Therapeutics Solutions; GM, Hitachi Chemical Regenerative Medicine Business Sector

Speakers:

David Lennon, Ph.D., President, AveXis
Ron Philip, SVP, Head of Global Commercial, Spark Therapeutics
Pascal Touchon, SVP and Global Head, Cell and Gene, Novartis Oncology

10:15am – 10:45am | MORNING BREAK
Sponsored by PeproTech and WiCell

10:45am – 12:00pm | CONCURRENT TRACKS

SPOTLIGHT SESSION: COMMERCIALIZING GENE THERAPIES FOR HEMOPHILIA *{Ballroom 1}*

10:45am – 11:15am

Compared to traditional methods for managing hemophilia, gene therapy holds the promise of greatly improving patient lives. This session brings together drug developers and manufacturers working to bring curative, state-of-the-art gene-based therapies to patients. The panel will explore opportunities and challenges of preclinical and clinical AAV programs for hemophilia.

Chair:

Jerry Keybl, Ph.D., Head of Cell and Gene Therapy Manufacturing Franchise, MilliporeSigma

Speakers:

Jonathan Garen, Chief Business Officer, uniQure
Dan Levin, Global Commercial Development and Hemophilia Marketing Lead, Pfizer
Sandy Macrae, Ph.D., President and CEO, Sangamo Therapeutics

COMPANY PRESENTATIONS *{Ballroom 1}*

11:15am	Atara Biotherapeutics
11:30am	bluebird bio
11:45am	MolMed

COMPANY PRESENTATIONS *{Ballroom 2}*

10:45am	ReNeuron
11:00am	Agilis Biotherapeutics
11:15am	Immusoft
11:30am	Capricor Therapeutics
11:45am	BlueRock Therapeutics

12:00pm – 1:15pm | LUNCH
Sponsored by Dark Horse Consulting

1:15pm – 3:30pm | CONCURRENT TRACKS

PANEL: NAVIGATING ACCEPTANCE, UPTAKE AND AFFORDABILITY ACROSS THE LIFECYCLE *{Ballroom 1}*
1:15pm – 2:15pm
Sponsored by Evidera
Regenerative and advanced therapies are now entering a changing and increasingly restrictive global environment. There is movement towards value assessment across the lifecycle by both regulators, HTA agencies and payers in the U.S. and EU. There is also growing focus on the affordability of costly therapies. Success in the emerging environment requires focus on value demonstration across the entire lifecycle from the earliest stages of development thru post-market differentiation as new therapies become available. This session will bring together regulators, manufacturers and payers to discuss what "good" looks like in the new global healthcare environment.

Chair:

Eric Faulkner, VP, Precision and Transformative Technology Solutions, Value Demonstration, Access and Commercial, Evidera

Speakers:

John Doyle, Dr.P.H., SVP and General Manager, Enterprise Solutions, Real-World and Analytics Solutions, IQVIA
Louis Jacques, M.D., Chief Clinical Officer, ADVI
Pamela Keith, Director, Oncology Reimbursement, Access, and Value Marketing, Juno Therapeutics, a Celgene company
Ron Philip, SVP, Head of Global Commercial, Spark Therapeutics
Pilar Pinilla-Dominguez, Senior Scientific Adviser, National Institute for Health and Care Excellence (NICE)
Richard Powell, M.D., Chief Medical Officer, MedPOINT Management

COMPANY PRESENTATIONS *{Ballroom 2}*

1:15pm	Frequency Therapeutics
1:30pm	Opsis Therapeutics
1:45pm	B-MoGen Biotechnologies
2:00pm	Synpromics

COMPANY PRESENTATIONS *{Ballroom 1}*

2:15pm	Homology Medicines
2:30pm	Abeona Therapeutics
2:45pm	uniQure
3:00pm	AVROBIO
3:15pm	Sangamo Therapeutics
3:30pm	Solid Biosciences

COMPANY PRESENTATIONS {Ballroom 2}

2:15pm	Zelluna Immunotherapy
2:30pm	CARISMA Therapeutics
2:45pm	Krystal Biotech
3:00pm	Fibrocell
3:15pm	Thrive Bioscience
3:30pm	MaxCyte

3:45pm – 4:00pm | AFTERNOON BREAK
Sponsored by PeproTech and WiCell

4:00pm – 6:00pm | CONCURRENT TRACKS

PANEL: OPPORTUNITIES AND CHALLENGES IN RARE DISEASE *{Ballroom 1}*
4:00pm – 5:00pm
Sponsored by Cell and Gene Therapy Catapult
Cell and gene therapies have moved from promise to reality in 2018, with rare diseases often in the vanguard of these remarkable new living medicines. Many firms have built substantial franchises in rare diseases, but there is still great

COMPANY PRESENTATIONS {*Ballroom 2}*

4:00pm	Organovo
4:15pm	DiscGenics
4:30pm	Histogenics
4:45pm	Dyno Therapeutics

unmet need and the potential to displace and disrupt existing therapies. This panel will explore the opportunities and challenges in rare diseases across cell and gene therapy modalities as these therapies come to market; exploring technical challenges, licensing, adoption and reimbursement in healthcare systems and public or patient attitudes to this medical revolution.

Chair:

Keith Thompson, CEO, Cell and Gene Therapy Catapult

Speakers:

Max Colao, Chief Commercial Officer, Abeona Therapeutics

Geoff MacKay, President and CEO, AVROBIO

Matthew Patterson, CEO, Audentes Therapeutics

Alvin Shih, CEO, Enzyvant

COMPANY PRESENTATIONS *{Ballroom 1}*

5:00pm	Adverum Biotechnologies
5:15pm	Nightstar Therapeutics
5:30pm	Athersys
5:45pm	BioTime

COMPANY PRESENTATIONS {*Ballroom 2}*

5:00pm	Sentien Biotechnologies
5:15pm	Unicyte
5:30pm	Miromatrix
5:45pm	American Gene Technologies

6:00pm | PARTNERING CLOSES

6:30pm – 9:30pm | GALA RECEPTION
Sponsored by BlueRock Therapeutics and CCRM

DAY 2

PARTNERING FORUM

THURSDAY | OCTOBER 4, 2018
ESTANCIA LA JOLLA HOTEL & SPA

6:45am – 9:15am | REGISTRATION AND BREAKFAST
Sponsored by KBI Biopharma

7:15am – 8:45am | CONCURRENT WORKSHOPS

PATIENT AND PUBLIC ATTITUDES TOWARD GENE THERAPY WORKSHOP
Magnolia Room | 7:15am – 8:45am

7:15am – 7:20am | Welcome Remarks
Speaker:
Dena Ladd, Executive Director, Missouri Cures

7:20am – 7:40am | Perceptions and Misconceptions: What is the current landscape?
This discussion will address existing patient and public attitudes toward gene therapy, and examine gaps in awareness and understanding as well as ideas on tools to fill them going forward.
Speakers:
Michelle Berg, VP, Patient Affairs and Community Engagement, Abeona Therapeutics

READINESS STRATEGIES FOR CELL THERAPY COMMERCIAL MANUFACTURING WORKSHOP
Learning Theater | 7:15am – 8:45am
Lead Sponsor: Hitachi Chemical Advanced Therapeutics Solutions

After several decades of steady progress in cancer research, the cell-based immunotherapy approach has shown significant potential with two-major milestone CAR-T cell therapies recently being approved. While multiple clinical trials are underway and being planned, the industry continues to be faced with challenges on how to commercialize, and to properly prepare for sustainable commercialization of these promising therapies. Participants in this moderated workshop will discuss considerations for readiness strategies to help prepare and

EVOLVING THE SUPPLY CHAIN FOR ADVANCED THERAPIES WORKSHOP
Ballroom 2 | 7:15am – 8:45am
Sponsored by World Courier

This session will use brief presentations to highlight the lessons learned within the supply chain, viewed through an industrial, academic, CMO and orchestration lens. These perspectives will then be used to discuss opportunities for the advanced therapy industry to learn from the past and create commercially viable logistics platforms.

This workshop will highlight topics such as how to manage:
• Cost by reducing complexity
• Vein to vein journey as a single inter-related system

Susan Sikora, Program Director, ARM Foundation for Cell and Gene Medicine

7:40am – 8:20am | Successful Partnerships Between Industry and Patient Organizations
This presentation will explore case studies on how industry can successfully partner with the patient communities they serve, and how the patient perspectives can be integrated into clinical research and educational programs.
Speakers:
Amy Fisher, Patient Advocacy Lead, Spark Therapeutics
Kristin Smedley, President and Co-Founder, Curing Retinal Blindness Foundation
Kimberly Trant, Director, Head of Patient Advocacy and Engagement, Audentes Therapeutics

8:20am – 8:40am | Externally-led Patient-Focused Drug Development Meetings: What are they and what are the benefits?
This portion of the workshop will discuss how these meetings are organized and the systematic approach they provide to ensure that patient's experiences, perspectives, needs and priorities are meaningfully incorporated into drug development and evaluation.
Speaker:
Jen Farmer, Executive Director, Friedreich's Ataxia Research Alliance

8:40am – 8:45am | Closing Remarks
Speaker:
Dena Ladd, Executive Director, Missouri Cures

innovate for global, scalable, cost efficient cell therapy commercial manufacturing and manufacturing platforms.

7:15am – 7:20am | Welcome Remarks
Speaker:
Robert Preti, Ph.D., President and CEO, Hitachi Chemical Advanced Therapeutics Solutions; GM, Hitachi Chemical Regenerative Medicine Business Sector

7:20am – 7:45am | Section 1: Commercialization
This section of the workshop will discuss what strategies are being considered when commercializing cell therapies and current and future challenges faced to properly prepare for sustainable commercialization of these promising therapies.
Chair:
Sanjin Zvonić, Ph.D., Senior Director, Business Leader, Clinical and Commercial Manufacturing, Hitachi Chemical Advanced Therapeutics Solutions
Speaker:
Usman "Oz" Azam, M.D., President and CEO, Tmunity Therapeutics

7:45am – 8:10am | Section 2: Global Footprint
This section of the workshop will discuss challenges related to global supply of cell therapies including global footprint and centralization strategies.
Chair:
Kazuchika Furuishi, Ph.D., Deputy General Manager, Hitachi Chemical Co.
Speakers:
Devyn Smith, Ph.D., Chief Strategy Officer and Head of Operations, Sigilon Therapeutics
Kimberly Freeman, VP, Commercial Strategy and Planning, Adaptimmune

8:10am – 8:35am | Section 3: Technology
The success of cell therapies to date has been enabled by an extraordinary technological evolution over many decades. While the commercial cell therapy industry is in its infancy, we should expect an accelerated emergence of commercial manufacturing technology in the coming years. For therapeutics in the clinic today and in the future, what should a technology readiness strategy consider?
Chair:

• Scale up/out by utilizing a logistics platform early in the development pathway

7:15am – 7:25am | Welcome Remarks
Chair:
Simon Ellison, Cell and Gene Therapy Service Director, World Courier

7:25am – 7:35am | Supply Chain Lessons from Marketed Autologous Therapies
Speaker:
Sven Kili, M.D., Principle, Sven Kili Consulting

7:35am – 7:45am | The Rise of the Academic Medical Center in the Cell and Gene Therapy Supply Chain
Speaker:
James Kovach, M.D., Director, Entrepreneurship and Innovation, UC Davis Health

7:45am – 7:45am | A Path to Commercially Viable Cell and Gene Therapies
Speaker:
Thomas Fellner, Ph.D., Head of Cell and Gene Therapy, Lonza

7:55am – 8:05am | Reducing Risks, Costs and Time in Personalized Supply Chains
Speaker:
Amy DuRoss, CEO, Vineti

8:05am – 8:45am | Moderated Discussion with All Speakers

David Kneen, VP, Cell Therapy, Invetech
Speakers:
Claudia Zylberberg, Ph.D., CEO, Akron Biotech
Gil Van Bokkelen, Ph.D., Chairman and CEO, Athersys

8:35am – 8:45am | Closing Remarks
Speaker:
Robert Preti, Ph.D., President and CEO, Hitachi Chemical Advanced Therapeutics Solutions; GM, Hitachi Chemical Regenerative Medicine Business Sector

9:00am | GENERAL SESSION AND PARTNERING OPENS

9:00am – 9:15am **OVERVIEW OF THE ALLIANCE FOR REGENERATIVE MEDICINE'S INITIATIVES** *{Ballroom 1}*
Speaker:
Janet Lambert, CEO, Alliance for Regenerative Medicine (ARM)

PANEL: LARGE PHARMA/BIOTECH'S LEADERSHIP ROLE – SUPPORTING THE COMMERCIAL SUCCESS OF CELL AND GENE THERAPIES *{Ballroom 1}*
9:15am – 10:15am
The era of cell and gene therapy has arrived. This panel will discuss large biopharma's role and likely impact in developing and commercializing new and disruptive technologies globally.
Chair:
Gbola Amusa, M.D., Partner, Director of Research and Head of Healthcare Research, Chardan
Speakers:
Brian Bronk, Ph.D., Head of External Innovation, Rare Diseases, Sanofi
Martin Golden, SVP, Head of Global Marketing Strategy, Astellas Pharma
Gabriele Proetzel, Ph.D., Director, Regenerative Medicine, Takeda Pharmaceuticals
Bob Smith, SVP, Global Gene Therapy Business, Pfizer

COMPANY PRESENTATIONS *{Ballroom 2}*

9:15am	Nohla Therapeutics
9:30am	Cellerant Therapeutics
9:45am	Terumo BCT
10:00pm	StemBioSys

10:15am – 10:45am | MORNING BREAK
Sponsored by PeproTech and WiCell

10:45am – 12:00pm | CONCURRENT TRACKS

FEATURED TALK: FDA'S EFFORTS TO ADVANCE THE DEVELOPMENT AND APPROVAL OF CELLULAR AND GENE THERAPIES *{Ballroom 1}*
10:45am – 11:00am
Speaker:
Peter Marks, M.D., Ph.D., Director, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration (FDA)

PANEL: RMAT REGULATORY CONVERGENCE: STARTING THE CONVERSATION *{Ballroom 1}*
11:00am – 12:00pm

COMPANY PRESENTATIONS {*Ballroom 2}*

10:45am	Regenerative Patch Technologies
11:00am	AGTC
11:15am	Fujifilm Cellular Dynamics
11:30am	Precision BioSciences
11:45am	Caribou Biosciences

This session will engage regulators and other industry leaders in an interactive conversation focused on expediting regulatory convergence as well as identifying actions that can be taken to further facilitate global convergence. Topics to be covered include ongoing efforts and priorities for regulatory convergence by focus area including CMC, nonclinical and clinical.

Sponsored by Janssen R&D

Chair:

Melody Eble, Pharm.D., Director, Global Regulatory Affairs, Scientific Innovation Projects – Digital Technology and Regenerative Medicine, Janssen R&D

Speakers:

Antony Appleyard, Ph.D., Technical Director Regulatory, Diamond Biopharm

Peter Marks, M.D., Ph.D., Director, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration (FDA)

Yoshiaki Maruyama, Ph.D., Review Director, Office of Cellular and Tissue-based Products, Pharmaceuticals and Medical Devices Agency (PMDA)

Jiwen Zhang, Ph.D., President, Standards Coordinating Body (SCB); Executive Director, Regulatory Affairs, Tmunity Therapeutics

12:00pm – 1:15pm | LUNCH

Sponsored by Paragon Bioservices

1:15pm – 2:45pm | CONCURRENT TRACKS

SPOTLIGHT SESSION: TISSUE ENGINEERING AND ORGAN TRANSPLANTATION *{Ballroom 1}*

1:15pm – 1:45pm

Tissue engineering holds the promise to eliminating the organ transplant waiting list, where over 120,000 patients currently wait, but how close are real solutions? Commercially, tissue engineering products have been limited to acellular or relatively thin constructs that lack vasculature and the complexity of functional tissue. This panel will discuss how recent advancements in decellularization technology and biologic scaffolds are addressing the vascular challenge. The speakers will describe the current pipeline of functional tissue engineered products including whole organs, how advancements in cell therapy have accelerated the path to market, the manufacturing, cell sourcing and distribution considerations when commercializing tissue engineering products and how recent regulatory programs – including RMAT – have fast-tracked the process.

Chair:

Jeff Ross, Ph.D., CEO, Miromatrix Medical

Speakers:

Jim McGorry, CEO, Biostage

Jason Wertheim, M.D., Ph.D., Associate Professor of Surgery – Organ Transplantation, Northwestern University

COMPANY PRESENTATIONS *{Ballroom 1}*

1:45pm	MeiraGTx
2:00pm	Adaptimmune

COMPANY PRESENTATIONS *{Ballroom 2}*

1:15pm	Monarch Biosciences
1:30pm	Semma Therapeutics
1:45pm	Sigilon Therapeutics
2:00pm	ViaCyte
2:15pm	Caladrius Biosciences
2:30pm	SCM Lifescience

2:15pm Iovance Biotherapeutics
2:30pm Mustang Bio

2:45pm – 3:15pm | AFTERNOON BREAK
Sponsored by PeproTech and WiCell

3:15pm – 5:30pm | CONCURRENT TRACKS

PANEL: NEXT GENERATION CELL AND GENE THERAPY IN ONCOLOGY *{Ballroom 1}*
3:15pm – 4:15pm
With the recent approvals of the first cell and gene therapies in oncology, this panel will discuss what comes next. Focus will include improvements in manufacturing processes and supply chain, lessons learned in regulatory pathways including program prioritization decision making and clinical development approaches to highlight disease population choices, study design including endpoints, utilization of biomarkers and surrogates, comparator groups, study duration and safety assessments.

Chair:

Timothy Schroeder, Founder and CEO, CTI Clinical Trial and Consulting

Speakers:

Maria Fardis, Ph.D., President and CEO, Iovance Biotherapeutics
Rachel Haurwitz, Ph.D., President and CEO, Caribou Biosciences
Sanjaya Singh, Ph.D., VP and Global Head, Janssen BioTherapeutics, Janssen R&D, Janssen Pharmaceutical Companies of Johnson & Johnson
Jeffrey Walsh, Chief Financial and Strategy Officer, bluebird bio

COMPANY PRESENTATIONS *{Ballroom 2}*

3:15pm Cynata Therapeutics
3:30pm Regenerex
3:45pm TikoMed
4:00pm MEDIPOST America

COMPANY PRESENTATIONS {*Ballroom 1}*

4:15pm Mesoblast
4:30pm Healios
4:45pm MiMedx
5:00pm Flexion Therapeutics
5:15pm Vericel

COMPANY PRESENTATIONS {*Ballroom 2}*

4:15pm Aegle Therapeutics
4:30pm Cell Medica
4:45pm Longeveron
5:00pm Cells for Cells
5:15pm Orbsen Therapeutics

5:30pm | PARTNERING CLOSES

DAY 2 PF

PUBLIC FORUM

THURSDAY I OCTOBER 4, 2018
SANFORD CONSORTIUM FOR REGENERATIVE MEDICINE

5:45pm | PUBLIC FORUM OPENS

6:00pm – 6:45pm **FEATURED PRESENTATION: REJUVENATING STEM CELL FUNCTION AND MUSCLE STRENGTH: NO PAIN, NO GAIN!**

Featured Speaker:

Helen M. Blau, Ph.D., Donald E. and Delia B. Baxter Foundation Professor; Director, Baxter Laboratory for Stem Cell Biology, Stanford University

6:45pm – 7:30pm | RECEPTION AND NETWORKING
Sponsored by Mesoblast

7:30pm | PUBLIC FORUM CLOSES

DAY 3

SCIENTIFIC SYMPOSIUM

FRIDAY | OCTOBER 5, 2018
SALK INSTITUTE FOR BIOLOGICAL STUDIES

7:00am – 8:00am | REGISTRATION AND BREAKFAST
Sponsored by Polyplus-transfection

8:00am | GENERAL SESSION

8:00am – 8:15am **WELCOME REMARKS**

Speaker:

Alysson Muotri, Ph.D., Chair, Scientific Symposium Steering Committee; Co-Director, Stem Cell Program; Professor, Department of Pediatrics and Cellular and Molecular Medicine, UC San Diego

8:15am – 8:55am **SELF-ORGANIZING SYNTHETIC HUMAN EMBRYOS AND ORGANOIDS TOWARDS CURING HUNTINGTON'S DISEASE**

Sponsored by Homology Medicines

Keynote Speaker:

Ali Brivanlou, Ph.D., Robert and Harriet Heilbrunn Professor; Head, Laboratory of Stem Cell Biology and Molecular Embryology, The Rockefeller University

8:55am – 10:15am **PANEL: EX VIVO GENE THERAPY: USING BLOOD STEM CELLS TO TREAT GENETIC DISORDERS**

Gene therapy is now a therapeutic reality for some terminal or severely disabling disorders. Ex vivo hematopoietic stem cell gene therapy has the critical advantage to turn the cells into widespread delivery vehicles to obtain stable and sustained expression of a defective protein in all appropriate tissues after a single systemic infusion. This session will highlight the potential of this approach in different disorders illustrating the technologies for hematopoietic stem cell gene-correction and the engagement of stem cells to prevent tissue degeneration.

Chair / Introduction By:

Stephanie Cherqui, Ph.D., Associate Professor, Department of Pediatrics, Division of Genetics, UC San Diego

Clinical Translation of Hematopoietic Stem Cell Gene Therapy for Cystinosis, Mechanism of Action and Other Applications

Stephanie Cherqui, Ph.D., Associate Professor, Department of Pediatrics, Division of Genetics, UC San Diego

Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy

Florian Eichler, M.D., Director, Center for Rare Neurological Diseases; Associate Professor, Neurology, Harvard Medical School; Assistant in Neurology, Massachusetts General Hospital

Genome Editing of Hematopoietic Stem Cells to Treat Human Genetic Diseases

Matthew Porteus, M.D., Ph.D., Associate Professor, Pediatrics – Stem Cell Transplantation and Regenerative Medicine, Stanford University

10:15am – 10:40am | MORNING BREAK
Sponsored by PeproTech and WiCell

10:15am – 10:40am | POSTER VIEWING
Sponsored by Brammer Bio

10:40am – 12:00pm **PANEL: ADVANCED THERAPIES FOR SKELETAL MUSCLES**

This panel will focus on the central role of muscle stem cells (MuSC) in skeletal muscle homeostasis and repair. Speakers will discuss how the MuSC compartment changes in the context of aging and muscle degenerative diseases and how they interact with the tissue microenvironment, as these findings reveal novel potential tools/targets to promote MuSC function and tissue repair. In addition, the panel will also discuss novel translational approaches to generate myogenic progenitors for cell-based therapies for muscle diseases.

Chair / Introduction By:

Alessandra Sacco, Ph.D., Associate Professor, Development, Aging and Regeneration Program; Associate Dean of Curriculum, Graduate School of Biomedical Sciences, Sanford Burnham Prebys Medical Discovery Institute

Focus on Muscle Stem Cell Aging

Andrew Brack, Ph.D., Associate Professor, Orthopaedic Surgery Research, Department of Orthopaedic Surgery, UC San Francisco

From Skin to Skeletal Muscle: A Potential for Autologous Transplantation in Muscular Dystrophies

Rita Perlingeiro, Ph.D., Lillehei Professor in Stem Cell and Regenerative Cardiovascular Medicine, Lillehei Heart Institute; Professor of Medicine, Cardiovascular Division, University of Minnesota

Cellular and Molecular Responses of Skeletal Muscle to Homeostatic Perturbations in Health and Disease

Pier Lorenzo Puri, M.D., Ph.D., Professor, Development, Aging and Regeneration Program, Sanford Burnham Prebys Medical Discovery Institute

12:00pm – 1:15pm | LUNCH
Sponsored by the Sanford Stem Cell Clinical Center at UC San Diego Health

12:00pm – 1:15pm | POSTER VIEWING
Sponsored by Brammer Bio

1:15pm – 2:35pm

PANEL: GENE THERAPY FOR NEURODEGENERATIVE DISEASES

This session will provide an in-depth update on clinical and preclinical programs of AAV9 gene therapy for spinal muscular atrophy, antisense oligonucleotide therapy for ALS and growth factor gene therapy for Alzheimer's disease.

Chair / Introduction By:

Mark Tuszynski, M.D., Ph.D., Director, Center for Neural Repair; Professor, Department of Neurosciences, UC San Diego

Translation of Gene Therapeutics in Neurological and Neuromuscular Diseases

Brian Kaspar, Ph.D., Principal Investigator, Center for Gene Therapy; Associate Professor, Department of Pediatrics and Department of Neuroscience, The Ohio State University; Chief Scientific Officer, AveXis

Making Sense of Antisense: ASO Therapy Development for C9orf72 Amyotrophic Lateral Sclerosis and Frontotemporal Dementia

John Ravits, Ph.D., Professor of Clinical Neuroscience, UC San Diego

Growth Factor Gene Therapy for Alzheimer's Disease

Mark Tuszynski, M.D., Ph.D., Director, Center for Neural Repair; Professor, Department of Neurosciences, UC San Diego

2:35pm – 3:00pm | AFTERNOON BREAK
Sponsored by PeproTech and WiCell

2:35pm – 3:00pm | POSTER VIEWING
Sponsored by Brammer Bio

3:00pm – 4:20pm

PANEL: USING STEM CELLS TO STUDY NEUROPSYCHIATRIC DISORDERS

The ability to generate neural derivatives from accessible somatic cells from patients with mental disorders (and appropriate controls) is beginning to make these heretofore "mechanistically-unapproachable" complex conditions amenable to rigorous molecular and cellular interrogation. Links are emerging between psychopathology and dysregulation of synaptogenesis, dendritogenesis, cytoskeleton, channels, glial support and inflammation, to name some examples. This session will provide an update on progress in this emerging area. A key take-away will be an appreciation that stem cell modeling has allowed us to begin to gain

previously elusive insights into the potential cellular and molecular underpinnings of pathologies that manifest principally by abnormalities in behavior.

Chair / Introduction By:

Evan Snyder, M.D., Ph.D., Director, Center for Stem Cells and Regenerative Medicine; Professor, Human Genetics Program, Sanford Burnham Prebys Medical Discovery Institute

Modeling the Impact of Common and Rare Variants in Schizophrenia Using Stem Cells

Kristen Brennand, Ph.D., Associate Professor, Departments of Genetics and Genomics, Neuroscience and Psychiatry, Icahn School of Medicine, Mount Sinai; New York Stem Cell Foundation – Robertson Investigator

Engineering Brain Organoids for Understanding Human Brain Development and Diseases

Guo-Li Ming, M.D., Ph.D., Professor of Neuroscience, Perelman School of Medicine, University of Pennsylvania

Using Stem Cell Models to Study Bipolar Disorders and Neuroinflammation

Carol Marchetto, Ph.D., Senior Staff Scientist, Laboratory of Genetics, Salk Institute for Biological Studies

4:20pm – 5:00pm **GENE EDITING IN HUMAN EMBRYOS**

Sponsored by Homology Medicines

Keynote Speaker:

Kathy Niakan, Ph.D., Group Leader, The Francis Crick Institute

5:00pm – 6:30pm | NETWORKING RECEPTION

5:00pm – 6:30pm | POSTER VIEWING

Sponsored by Brammer Bio

6:30pm | SCIENTIFIC SYMPOSIUM CLOSES