Agenda
70+ PRE-RECORDED VIRTUAL COMPANY PRESENTATIONS

BESPOKE THERAPIES FOR ULTRA-RARE DISEASE WORKSHOP
Sponsored by Pfizer
This session will explore the recently formed National Institutes of Health (NIH)'s, Foundation for the NIH, FDA's and the biopharmaceutical industry's innovative public-private partnership to help individualized gene therapies reach patients despite their lack of commercial viability. Panelists will provide an overview of this initiative including how it will maximize benefits to patients, as well as explore the specific opportunities and challenges related to its success.

Chair: Bob Smith, Senior Vice President, Global Gene Therapy Business, Pfizer Inc.
Speakers:
Philip John (P.J.) Brooks, Ph.D., Program Director, Office of Rare Diseases Research, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)
Gregory LaRosa, Ph.D., Vice President, Head of Scientific Research, Rare Disease Research Unit, Pfizer Inc.
Peter Marks, M.D., Ph.D., Director, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration (FDA)
Courtney Silverthorn, Ph.D., Associate Vice President, Research Partnerships, Foundation for the National Institutes of Health (FNIH)

BIDEN ADMINISTRATION PLANS FOR LIFE SCIENCE INNOVATION
Chair: Janet Lambert, CEO, Alliance for Regenerative Medicine (ARM)
Speaker: Carrie Wolinetz, Ph.D., Deputy Director for Health and Life Sciences, White House Office of Science and Technology Policy

DOING BUSINESS IN JAPAN – AN EVOLVING MARKET WORKSHOP
Promoting Harmonization and Commercialization of Regenerative Medicines Among Asian Countries/Regions
In Partnership with the Forum for Innovative Regenerative Medicine (FIRM)
The Asia Partnership Conference of Regenerative Medicine (APACRM), is aimed at harmonizing and optimizing regulations among Asian countries and regions. FIRM has organized the APACRM since 2018 and has closely collaborated with industrial groups such as the China Medicinal Biotech Association (CMAA) in China; Association of Biotechnology Led Enterprises (ABLE) in India; Council for Advanced Regenerative Medicine (CARM) and Strategic Center for Regenerative Medicine (SCRM) in Korea; Singapore Association of Pharmaceuticals Industries (SAPI) in Singapore; and Biotechnology and Pharmaceutical Industries Promotion Office (BPIPO) in Taiwan. This session will focus on prospects and challenges for or against regenerative medicine to promote harmonization and optimization of the regulations on regenerative medicine products among Asian countries and regions.

Chair: Masayuki (Max) Nomura, Ph.D., Chair, International Affairs Committee, Forum for Innovative Regenerative Medicine (FIRM)
Opening Remarks: Kozo Saiki, Director, Bio-Industry Division, Ministry of Economy, Trade and Industry (METI)
**REGISTRATION AND BREAKFAST**  
Sponsored by KBI Biopharma

**CONCURRENT WORKSHOPS**

**WHAT’S NEXT FOR ADVANCED THERAPIES WORKSHOP**
*BlueRock Therapeutics Ballroom*

This workshop will feature leading executives in the ATMP space who will discuss a number of topics including recent advancements in clinical trials, going from treating rare diseases to prevalent conditions, and implementing next-generation gene-editing technologies. The session will also touch upon resolving manufacturing and talent bottlenecks as the sector moves towards commercialization, as well as the evolution of reimbursement payment models.

**Chair:** Anshul Mangal, President, Project Farma and Precision ADVANCE  
**Speakers:**  
- Phil Cyr, Senior Vice President, Customer Solutions, Precision Value & Health  
- Palani Palaniappan, Ph.D., Executive Vice President and Chief Technology Officer, Aruvant Sciences  
- Derrell Porter, M.D., Founder and CEO, Cellevolve  
- RA Session II, Founder, President and CEO, Taysha Gene Therapies  
- Claudia Zylberberg, Ph.D., Founder and Executive Chair, Akron Biotech

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**IN-PERSON PROGRAM AND LIVESTREAM**

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

- **7:15 – 8:45 am**  
  **CONCURRENT WORKSHOPS**

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**FEATURED FIRESIDE CHAT: FDA PERSPECTIVE**

**Chair:** Janet Lambert, CEO, Alliance for Regenerative Medicine (ARM)  
**Speaker:** Peter Marks, M.D., Ph.D., Director, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration (FDA)

**FROM CONCEPT TO COMMERCIALIZATION IN LIVING THERAPEUTICS: HINDSIGHT IS 20/20 WORKSHOP**

*Sponsored by Charles River Laboratories*

Challenges for developing a cell therapy product evolve and shift, and reducing these risks requires experience and expertise to execute around them. From discovery through late-phase clinical trials and into commercial manufacturing, different challenges and competing priorities are ever present – from coordinating multiple CROs/CDMOs, managing clinical operations effectively, and overcoming supply chain and logistic hurdles, all while keeping in line with regulatory standards. This discussion will take a look at these and many more real-world challenges and focus on the strategies involved with mitigating risks along the way while reducing the amount of lag time involved with bringing a cell therapy product to market.

**Chair:** Matthew Hewitt, Ph.D., Senior Director, Scientific Solutions Cell and Gene Therapy, Charles River Laboratories  
**Speakers:**  
- Douglas Doerfler, CEO, MaxCyte  
- Howard Federoff, M.D., Ph.D., President and CEO, Brooklyn ImmunoTherapeutics; Senior Advisor, Aspen Neuroscience  
- Kristin Yarema, Ph.D., Chief Commercial Officer, Atara Biotherapeutics
UNLOCKING THE POWER OF COLLABORATION TO FUEL THE INDUSTRY TRANSFORMATION FOR CELL & GENE THERAPIES WORKSHOP

Aviara Salon A

Sponsored by Pointellis, powered by EY

The groundwork for seamless cell and gene therapy (CGT) care delivery needs to be laid now. There are many stakeholders involved in the complexities of the CGT treatment journey that require a radically new approach from that of traditional mass-produced therapies. A complex balancing act of multi-stakeholder processes hinge on treatment advances, product availability, and the network’s ability to meet demand. And that’s before thinking about scaling CGT delivery to more than a few thousand patients. This workshop will bring together leading experts in managing interconnected supply and logistics networks, delivering superior patient experiences, manufacturing cell and gene therapies, and building world-class digital infrastructures.

Chair: Adlai Goldberg, Global Digital, Social and Commercial Innovation Life Sciences Leader, EY

Speakers:
- Sybil Danby, Senior Vice President, Business Development and Strategy, Center for Breakthrough Medicines
- Tamie Joeckel, Global Business Lead, Cell and Gene Therapy COE, ICON
- Orlando Serani, Program Lead Advanced Cellular Therapies Supply Chain, Johnson & Johnson
- Julia Tarasenko, Vice President, Commercial Operations, Europe and the Americas, Marken, a UPS Healthcare company

9:00 – 10:15 am GENERAL SESSION AND PARTNERING OPENS

9:00 am WELCOME REMARKS

AVROBIO Ballroom

Speakers:
- Janet Lambert, CEO, Alliance for Regenerative Medicine (ARM)
- Emile Nuwaysir, Ph.D., CEO, Ensoma; Chairman, Alliance for Regenerative Medicine (ARM); Chairman, BlueRock Therapeutics

9:15 am PLENARY SESSION: CELLULAR IMMUNOTHERAPY PIONEERS – LIQUID VS. SOLID TUMOR STRATEGIES

AVROBIO Ballroom

Chair: Usman “Oz” Azam, M.D., President and CEO, Tmunity Therapeutics

Speakers:
- Michael Dombeck, Chief Operating Officer, Arcellx
- Kanya Rajangam, M.D., Ph.D., Chief Medical Officer, Nkarta Therapeutics
- Devon Shedlock, Ph.D., Senior Vice President, Research and Development, Poseida Therapeutics
- Pascal Touchon, President and CEO, Atara Biotherapeutics

10:15 – 10:45 am MORNING BREAK Sponsored by Bone Therapeutics, Cytotheryx, Invetech and POMS

10:45 am – 12:00 pm CONCURRENT TRACKS

10:45 am LESSONS LEARNED: THE ROLE OF PATIENT ENGAGEMENT IN APPROVALS & PAYOR DISCUSSIONS

AVROBIO Ballroom

In a pre-recorded video, a mother of a child living with SMA who received Zolgensma will share her story and what she believes healthcare decision makers should include in their evaluations of gene therapies. ARM’s Paige Bischoff will then lead a live discussion with Novartis’ Amy Nicole Nayar on lessons emerging gene therapy companies can learn from Novartis’ experience incorporating the patient perspective in discussions with regulators, payors, and health technology assessors.

Chair: Paige Bischoff, Senior Vice President of Global Public Affairs, Alliance for Regenerative Medicine (ARM)

COMPANY PRESENTATIONS

BlueRock Therapeutics Ballroom
- 10:45 am Emendo Biotherapeutics
- 11:00 am Ensoma
WHERE ARE WE ON THE JOURNEY TO CURE SICKLE CELL DISEASE?

**AVROBIO Ballroom**

The sickle cell mutation was discovered in 1956. Unfortunately, the translation of knowledge into developing treatments has been disproportionately slow and elusive… until now. In the last five years, there has been a vast increase in the number of therapies in development and the prominence of sickle cell has grown on the agendas of professional societies and the public sector. How is this combination of attention and technology bringing us closer to a cure? Hear from leading experts on the latest cell and gene therapies aiming to address this devastating disease.

**Chair:** Jill Elliott, Vice President, Cell Therapy Commercialization, Novo Nordisk

**Speakers:**
- Edmond Chen, M.D., Vice President, Head of Clinical Development, Hematology, Editas Medicine
- Debbie Drane, Senior Vice President, Global Commercial Development and TA Strategy, CSL Behring
- Thomas Klima, Chief Commercial Officer, bluebird bio
- Sandy Macrae, Ph.D., President and CEO, Sangamo Therapeutics
- Amy Simon, M.D., Chief Medical Officer, Beam Therapeutics

**COMPANY PRESENTATIONS**

**AVROBIO Ballroom**

- 11:15 am: Oxford Biomedica
- 11:30 am: 4DMT
- 11:45 am: Helixmith

**COMPANY PRESENTATIONS**

**BlueRock Therapeutics Ballroom**

- 11:15 am: Orca Bio
- 11:00 am: Aldevron
- 11:45 am: InVitria
4:00 pm

**CURRENT REALITIES OF MAKE VS. BUY FOR CELL & GENE THERAPY MANUFACTURING**

*AVROBIO Ballroom*

This panel of leading experts will discuss key considerations and experiences related to the Make vs. Buy decision from a variety of perspectives. Speakers will debate whether those considerations are unique to today’s environment and how they might be different in the future. This session will solicit significant audience input to shape the discussion.

**Chair:** Kelvin Lee, Ph.D., Institute Director, National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL)

**Speakers:**
- Melissa Carpenter, Ph.D., Chief Scientific Officer, Regenerative Medicine, ElevateBio
- Thomas Fellner, Ph.D., Vice President, Global Head of Business Development, Account Management, and Program Management, Lonza Cell & Gene Technologies
- Nirupama (Rupa) Pike, Ph.D., Director, Enterprise Science and Innovation Partnerships, Global Corporate Accounts, Thermo Fisher Scientific
- Joe Tarnowski, Ph.D., Senior Vice President, Cell and Gene Therapy Platform, GlaxoSmithKline

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**COMPANY PRESENTATIONS**

*BlueRock Therapeutics Ballroom*

4:00 pm Cellatoz Therapeutics
4:15 pm Aspen Neuroscience
4:30 pm Labcorp
4:45 pm Mirus Bio

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**COMPANY PRESENTATIONS**

*AVROBIO Ballroom*

5:00 pm Amicus Therapeutics
5:15 pm Homology Medicines
5:30 pm AVROBIO
5:45 pm Orchard Therapeutics

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**COMPANY PRESENTATIONS**

*BlueRock Therapeutics Ballroom*

5:00 pm Forge Biologics
5:15 pm Life Edit Therapeutics
5:30 pm TreeFrog Therapeutics
5:45 pm GenEdit

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**6:00 pm**

**PROGRAM AND PARTNERING CLOSES**

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**6:30 – 9:30 pm**

**NETWORKING BASH** *Sponsored by Biopharma Excellence by PharmaLex, Cognate BioServices and L7 Informatics*
**Industrialization of Cell Therapy Manufacturing – Lessons Learned from Biologics Workshop**

*BlueRock Therapeutics Ballroom*

*Sponsored by Thermo Fisher Scientific*

The transformative impact that cell therapies have is evident from the observed efficacy of FDA-approved therapies to date. However, as an increasing number of drugs move from early discovery to commercialization, manufacturing innovations have moved to the forefront of the discussion. We have reached an inflection point that requires us to move from small scale, open workflows to robust, closed and automated manufacturing processes. In this session we will discuss and reflect on the various lessons that we have learned from biologics manufacturing and how they can be applied to advance the industrialization of the cell therapy manufacturing process. Join this session to hear from experts on:

- Innovation in “fit for purpose” closed cell therapy manufacturing equipment
- Controlling critical process parameters with digital integration utilizing a distributed control system (Delta V)
- Supply chain considerations
- Characterization and QC analytics for standardization

**Chair:** Betty Woo, Ph.D., Vice President and General Manager, Cell and Gene Therapy, Thermo Fisher Scientific

**Speakers:**

- Jerry Cacia, Chief Technical Officer, Graphite Bio
- Andy Lin, Ph.D., Principal Technical Development Leader, Pharma Technical Development, Genentech
- Sophia Sharp Donaldson, Executive Director, Supply Chain, Global Strategic Sourcing and Procurement, Kite, a Gilead company

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**Democratization of Transformative Cell & Gene Therapies Workshop**

*Aviara Salon A*

*Sponsored by IQVIA*

Recent years have seen dramatic success with gene therapy, even apparent cures for serious medical conditions (e.g., spino muscular atrophy, sickle cell disease). Yet, for several of the diseases being cured with gene therapy, the vast majority of patients who could potentially benefit reside in low- and middle-income countries (LMIC), where such therapies are not available. The reasons underlying inaccessibility are multifold, and both near-term and long-term solutions need to be devised and implemented by the regenerative medicine industry. Near and next-term solutions come from disparate areas from utilizing information technology with patient databases/registries, to improving efficiencies and cost of manufacturing and distribution. The challenges of gene therapy implementation in LMIC span several functional areas including education and communication, regulatory, clinical preparedness, and technical / logistical aspects. Looking at the longer-range and worldwide aspirations, one initiative called the Global Gene Therapy Initiative (GGTI) includes diverse stakeholders covering broad areas of expertise from all sectors. The GGTI has set an aspirational goal of launching Phase I gene therapy clinical trials in each of two LMIC, Uganda and India, by 2024.

**Chair:** Eugene Brandon, Ph.D., Head, Cell and Gene Therapy Translation, IQVIA

**Speakers:**

- Jennifer Adair, Ph.D., Associate Professor, Clinical Research Division, Stem Cell and Gene Therapy Program, Fred Hutchinson Cancer Research Center
- Boro Dropulic, Ph.D., Executive Director, Caring Cross
- Eric Faulkner, Vice President, Global Real World Evidence, Novartis Gene Therapies
- Mike McCullough, Chief Information Officer, National Marrow Donor Program (NMDP)/Be The Match
OVERCOMING CHALLENGES TO GENE THERAPY MANUFACTURING THROUGH STANDARDIZATION & AUTOMATION WORKSHOP

Aviara Salon B

Sponsored by Cell One Partners

Gene therapy clinical trials pose interesting challenges in GMP manufacturing. Plasmids and vectors often cannot be produced by the same vendors, may have different stabilities, and may require different storage conditions. Furthermore, some gene therapies require additional processing of the vector with target cells ex vivo. This expert-led session will explore innovations and best practices that are shortening timelines, lowering costs, and standardizing processes when manufacturing gene therapies.

Chair: Anthony Gringeri, Ph.D., Strategic Advisor, Cell One Partners

Speakers:
- David Backer, Chief Commercial Officer, Oxford Biomedica
- Michelle Berg, President, GMP Nucleic Acids, Aldevron
- Fabian Gerlinghaus, Co-Founder and CEO, Cellares
- Judith Koliwer, Ph.D., Lead Consultant Software, Körber Business Area Pharma

9:00 – 9:15 am GENERAL SESSION AND PARTNERING OPENS

9:00 am OVERVIEW OF ARM'S INITIATIVES

AVROBIO Ballroom

Speaker: Janet Lambert, CEO, Alliance for Regenerative Medicine (ARM)

9:15 – 10:15 am CONCURRENT TRACKS

9:15 am THE PAST, PRESENT & FUTURE OF GENE DELIVERY

AVROBIO Ballroom

Gene therapy holds promise for treating and, possibly, curing a wide range of diseases, including cancer, cystic fibrosis, heart disease, and diabetes. Since the first FDA approved gene therapy trial more than 30 years ago, the advances in gene therapy have rapidly evolved. However, challenges to delivering these life-saving therapies persist, many of which are bespoke to patients. Answers may be provided in technology, automation, and scientific advances.

Chair: Timothy Schroeder, Chairman and CEO, CTI Clinical Trial & Consulting Services

Speakers:
- Patrick Boyle, Ph.D., Head of Codebase, Ginkgo Bioworks
- Emma McBurney, Vice President, Business Development, Biogen
- Leslie Meltzer, Ph.D., Chief Medical Officer, Orchard Therapeutics
- Peter Nell, Ph.D., Chief Business Officer and Head of Therapeutics, Mammoth Biosciences
- Emile Nuwaysir, Ph.D., CEO, Ensoma; Chairman, Alliance for Regenerative Medicine (ARM); Chairman, BlueRock Therapeutics
- Devyn Smith, Ph.D., CEO, Arbor Biotechnologies

10:15 – 10:45 am MORNING BREAK

Sponsored by Bone Therapeutics, Cytotheryx, Invetech and POMS
10:45 am – 12:00 pm  CONCURRENT TRACKS

10:45 am  INNOVATIVE REGULATORY PATHWAYS FOR NEXT-GENERATION TREATMENTS
AVROBIO Ballroom

Cell and gene therapies are complex and the current regulations around them are also multifaceted – and constantly evolving. Regulatory agencies need to be involved throughout the development process to keep in lockstep and build their insights into the program. Ultimately, the goal is to create and execute an effective and efficient strategy that leaves nothing to chance. This session will feature discussion between leading developers of innovative therapies sharing insights about their key regulatory considerations and pathways to market.

Chair:  Mark Lane, Ph.D., Vice President, Development Consulting and Scientific Affairs, Biopharma Excellence by PharmaLex

Speakers:  Jennifer Dittman, Vice President, Regulatory Affairs, Generation Bio
           Rachelle Jacques, CEO, Enzyvant
           Jeff Ross, Ph.D., CEO, Miromatrix

11:15 am  STRATEGIES FOR MANAGING ACCESS & REIMBURSEMENT CHALLENGES
AVROBIO Ballroom

Chair:  Katy Spink, Ph.D., Chief Operating Officer and Managing Partner, Dark Horse Consulting Group

Speakers:  Eric Faulkner, Vice President, Global Real World Evidence, Novartis Gene Therapies
           Sarah Pitluck, Head, Global Pricing and Reimbursement, Spark Therapeutics
           Kristin Wolff, Vice President, Global Policy Strategy and U.S. Government Payer, bluebird bio

12:00 – 1:15 pm  LUNCH  Sponsored by Yposkesi

1:15 – 2:45 pm  CONCURRENT TRACKS

1:15 pm  UPDATE ON THE ARM FOUNDATION FOR CELL & GENE MEDICINE
AVROBIO Ballroom

Speakers:  Brett Kopelan, Chairman of the Board, ARM Foundation for Cell & Gene Medicine; Executive Director, debra of America
           Martha Rook, Ph.D., Treasurer, ARM Foundation for Cell & Gene Medicine; Chief Technical Operations Officer, Sigilon Therapeutics

1:25 pm  THE INFORMATION PATIENTS WANT ABOUT REGENERATIVE MEDICINE
AVROBIO Ballroom

Chair:  Brett Kopelan, Chairman of the Board, ARM Foundation for Cell & Gene Medicine; Executive Director, debra of America

COMPANY PRESENTATIONS

BlueRock Therapeutics Ballroom
10:45 am  Akron Biotech
11:00 am  MEDIPOST America

COMPANY PRESENTATIONS

BlueRock Therapeutics Ballroom
11:15 am  AGTC
11:30 am  ConeSight Therapeutics
11:45 am  SparingVision

COMPANY PRESENTATIONS

BlueRock Therapeutics Ballroom
1:15 pm  CTI Clinical Trial & Consulting
1:30 pm  ElevateBio
1:45 pm  GentiBio
Speakers: Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy (PPMD)
Leonard Valentino, M.D., President and CEO, National Hemophilia Foundation

COMPANY PRESENTATIONS
AVROBIO Ballroom
2:00 pm Sangamo Therapeutics
2:15 pm uniQure
2:30 pm Sigilon Therapeutics

COMPANY PRESENTATIONS
BlueRock Therapeutics Ballroom
2:00 pm Virica Biotech
2:15 pm Enzyvant Therapeutics
2:30 pm Sentien Biotechnologies

2:45 – 3:15 pm  AFTERNOON BREAK  Sponsored by Bone Therapeutics, Cytotheryx, Invetech and POMS

3:15 – 6:00 pm  CONCURRENT TRACKS

3:15 pm  PERSONALIZING CARDIOVASCULAR THERAPIES TO ADDRESS THE UNDERLYING CAUSES OF DISEASE
AVROBIO Ballroom

Cell and gene therapies are some of the most cutting-edge innovations in cardiovascular (CV) medicine and represent the next frontier of this therapeutic area. Even though CV cell and gene therapies have great promise to significantly improve clinical outcomes, it is still an emerging field, with a history of clinical trials yielding mixed results. Some of the key challenges in developing these therapies include understanding the true mechanisms of action, identifying the most effective cell types, dosing, CV clinical targets, and routes and frequency of administration. Other important questions about CV cell and gene therapy clinical development focus on how best to design clinical trials, what target populations to focus on, and what clinical development paradigms to use to successfully advance the field. This session includes leaders in the field who have already grappled with many of these questions, and have successfully addressed them to launch CV clinical development programs.

Chair: Monica Shah, M.D., Vice President and Head, Cell and Gene Therapy Center of Excellence, IQVIA

Speakers: Faraz Ali, CEO, Tenaya Therapeutics
Joachim Fruebis, Ph.D., Chief Development Officer, BlueRock Therapeutics
Jordan Lancaster, Ph.D., Co-founder and CEO, Avery Therapeutics
William “B.J.” Lehmann, Interim CEO, Athersys
Michael Scott, Ph.D., Vice President, Cell Therapy Medical Devices, Novo Nordisk

COMPANY PRESENTATIONS
AVROBIO Ballroom
4:15 pm Allogene Therapeutics
4:30 pm Mission Bio
4:45 pm Precigen
5:00 pm MaxCyte
5:15 pm Vor Biopharma
5:30 pm Poseida Therapeutics
5:45 pm Kuur Therapeutics

COMPANY PRESENTATIONS
BlueRock Therapeutics Ballroom
4:15 pm Sensorion
4:30 pm Likarda
4:45 pm Tenaya Therapeutics
5:00 pm Vineti
5:15 pm Rejuvenate Bio
5:30 pm Avery Therapeutics
5:45 pm Vascugen

6:00 pm  PROGRAM AND PARTNERING CLOSES
7:15 am DELIVERING THE INNOVATION OF GENE THERAPY TO PATIENTS THROUGH STRATEGIC COLLABORATIONS WORKSHOP

Aviara Salon B  
Sponsored by CSL Behring

This workshop will explore case studies where strategic collaborations have been the pathway of choice for gene therapy innovators to ensure an optimal route to commercialization. Leading executives in the gene therapy sector will discuss the rationale of these strategic partnerships along with key learnings for successful implementation.

Chair: Debbie Drane, Senior Vice President, Global Commercial Development and TA Strategy, CSL Behring

Speakers:
- Lawrence Kau, Global Commercial Strategy Lead, Spark Therapeutics
- Josh Leeman, Ph.D., Vice President, Business Development, Search and Evaluation, uniQure
- Adam Simpson, Ph.D., Head, Gene Therapies BD&L, Novartis Institutes for BioMedical Research

9:00 am ARM GROW INTERNSHIP UPDATE

AVROBIO Ballroom

Speakers:
- Ifeoluwa Awoleye, ARM GROW Intern, Prevail Therapeutics
- Rachelle Jacques, CEO, Enzyvant
- Janet Lambert, CEO, Alliance for Regenerative Medicine (ARM)
- Jamila Ritter, ARM GROW Intern, Amicus Therapeutics

9:15 am SYNTHEtic BIOLOGY APPLICATIONS IN CELL & GENE THERAPY

AVROBIO Ballroom

Chair: Robert Deans, Ph.D., Chief Scientific Officer, Synthego

Speakers:
- Timothy Lu, M.D., Ph.D., Co-founder and CEO, Senti Biosciences
- Ali Nahvi, Ph.D., Head of New Therapeutic Modalities, Spark Therapeutics

9:45 am WHAT CELL & GENE THERAPY INVESTORS ARE LOOKING FOR NOW

AVROBIO Ballroom

Chair: Phil Vanek, Ph.D., Chief Technology Officer, Gamma Biosciences

Speakers:
- Gbola Amusa, M.D., Partner and Chief Scientific Officer, Chardan
- Chris Garabedian, Chairman and CEO, Xontogeny
- Ed Hurwitz, Managing Director, MPM Capital
- Deborah Palestrant, Ph.D., Partner and Head of 459, SAM Ventures

COMPANY PRESENTATIONS

BlueRock Therapeutics Ballroom

9:15 am Mammoth Biosciences
9:30 am Humacyte
DOsing & Redosing in Gene therapy  
AVROBIO Ballroom

Since the earliest days of gene therapy, dosing has been a key pivotal issue to balance with tremendous benefit of this technology with its inherent risks. Today, understanding considerations of dosing across diverse patient populations for which we intend treatment is much improved, but still requires empiric trials. With a considerable number of key questions still to be answered, our goal should be to collectively and comprehensively explore issues in dosing, and ultimately, re-dosing of patients. Are we doing enough as an industry to confidently employ dosing strategies for gene therapies?

Chair: Chris Mason, M.D., Ph.D., Chief Scientific Officer, AVROBIO

Speakers: Eric Crombez, M.D., Chief Medical Officer, Ultragenyx Gene Therapy  
Peter Francis, M.D., Ph.D., Chief Scientific Officer, Ophthalmology Therapeutic Area Head, 4D Molecular Therapeutics  
Timothy Miller, Ph.D., CEO, President and Co-Founder, Forge Biologics  
Nicole Paulk, Ph.D., Professor, AAV Gene Therapy, University of California, San Francisco

COMPANY PRESENTATIONS  
BlueRock Therapeutics Ballroom

11:00 am Aspect Biosystems
11:15 am Trailhead Biosystems
11:30 am Stemson Therapeutics
11:45 am Synthego