NEXT GENERATION GENE THERAPEUTICS

NOVEMBER 14-16, 2024 | Philadelphia, PA

SPONSORS

DAY-1	NOVEMBER 14, 2024	THURSDAY

Registrations
Introduction to the conference "**Next Generation Gene Therapeutics**"

KEYNOTE

Next Wave of Gene Editing Therapies
Devyn M. Smith, Chief Scientific Officer, Arbor Biotech, MA

Programmable and Autoregulated Viral Vectors for Novel Applications of Gene Therapy
Farzin Farzaneh, Chief Scientific Officer, ViroCell, UK

Coffee Break

The Concept of Innovation and Orchestration: Translating Cellular Therapies from Bench to Bedside
Khalid Shah, Professor, Harvard Medical School, Boston, MA

Creating a world leading innovation hub
Audrey Greenberg, CEO, Center for Breakthrough Medicnes

Leveraging Regulatory Changes to Accelerate Gene Therapy Development & Reduce Manufacturing Cost
Angela Johnson, Global Regulatory & Compliance Leader, Cytiva

Next generation of gene therapy delivery platform
Shira Orr, CEO & Co-Founder, Envoya Inc.

In Pursuit of Gene Therapy Treatment for Atherosclerosis
Paul Hermonat, Chief Executive Officer, Houston Gene Therapeutics

Networking Lunch

TBA
Albert Freedman, Freedman Counseling Associates

Innovative Gene Delivery Platforms for Therapeutics
Robert (Chih Hong) Lou, CTO, Carrigent Inc.

Hindsight is 2020: The Advancement of CpG-depleted AAV Vectors as an Industry Standard
Susan M. Faust PhD, CEO, NxGEN Vector Solutions

TBA
Mitchell Finer, CEO, Elevate Bio

TBA
Luke Yu, CEO, Biocytogen Inc.

Panel Discussion

Gene Therapy for the Generation of CAR-T Cells

Moderator:

About Panel:

In a nutshell, cell therapy has proven gene editing of immune cells can be extremely valuable modality to treat patients with unmet needs such as the approved CAR-T cell therapy products. The challenge is these are extremely complex to manufacture, costly, production scale is very low and thus access limited. The idea to simply deliver the gene in vivo is very attractive but now the cell therapy challenges have simply been replace by gene therapy challenges, primarily accurate and safe dosing. That said, 2 dozen companies are now exploring and clinical data should start reading out in next few years.

Coffee Break

TOPICS:

Application of CAR & CAR-T
Cell & Gene Therapy Bioprocessing
Advances in Cell & Gene Therapy Research

Enabling Allogeneic T cell-based Therapies: Scalable Stirred-tank bioreactor mediated manufacturing
Inbar Friedrich Ben-Nun, Cell and Gene Therapy Research and Development, Lonza Inc., MD

Engineered Nanomaterial-mediated Systemically Administered m-RNA-based Gene therapy Directed Exclusively to Cancer, Present Successes and Future Prospects
A. C. Matin, Professor, Stanford University School of Medicine, CA

Proteo-Lipid Vehicle Delivery of Nucleic Acid Therapeutics for Effective Payload Transport
John Lewis, Chief Executive Officer, Entos Pharmaceuticals, MA

TBA
Hubert Lam, Vice-President, Affini-T Therapeutics

TBA
John Leonard, Chief Executive Officer, Leading Edge Bio, CA

TBA
Bruce Dezube, Mustang Bio, Inc.

Speaking Slots Available

Welcome Reception

DAY-2	**NOVEMBER 15, 2024**	**FRIDAY**

Registrations

Panel Discussion

Moderator:

Gene Therapy for Rare Diseases

About Panel:

1. Challenges facing ultra-rare patients, academic centers, non-profits/bespoke projects today

2. Challenges with site/country selection, CTA approval and study site startup for rare disease indications

3. Challenges with GMP manufacturing, CMO selection, QC testing, costs and complexities, especially for startup enterprises targeting rare diseases

Coffee Break and refreshments

TOPICS: **Gene Therapy for Rare Diseases**

Identifying Novel Gene Therapies for Common Pathological Problems In Neurological, Ophthalmological and Rare Disorders
Shane Hegarty, Chief Scientific Officer & Co-Founder, AXONIS Therapeutics, Inc.

Gene Therapy for the MPS and ML II/III Disorders: Experience and Research Resources Moving Forward
Matthew Ellinwood, Chief Scientific Officer, National MPS Society, NC

Speaking Slots Available

TOPICS:
Commercialization Strategies and Market Analysis
Investment Trends in Gene Therapy Sector
Vector Products & Delivery Platforms

Prevalence of errors in lab-made plasmids across the globe
Kristofer Mussar, CEO, Vector Builder Inc.

A New Era for RNA Pharmaceuticals – What comes next?
Charles Jones, Senior Director, mRNA Commercial Strategy & Innovation, Pfizer

Developing Intellectual Property Strategies for Gene Therapy Products
Kevin MacDonald, Associate, Wolf, Greenfield & Sacks, P.C.

Speaking Slots Available

Networking Lunch

TOPICS:
Gene Editing Tools and Technologies – CRISPR, ZFP
Current Gene Therapy Progress: Targets and Challenges
Gene Therapy Bioprocessing

Validation of a multiplex RT-qPCR Assay for the Detection of miRNAs in Human Samples
Jeffrey Alex Washburn, Labcorp Inc.

TBA
Fernando Aleman, Founder & CSO, Navega Therapeutics, CA

Finding Your Footing: How to Balance the Toxicity, Specificity and Feasibility of Vector Designs
Andrew Murphy, VP, Early Research, Kriya Therapeutics

TBA
Nidhi Kotecha, Gates Institute

The CMC Solution to Personalized mRNA Medicines
Molly McGlaughlin, CEO, Kudo Biotechnology

TBA
Suman Jangid, BridgeBio

Industrial Scale Manufacture of ProtoNK Cells Using 3-D Bioreactor
Shi-Jiang (John) Lu, President and CEO, HebeCell Corporation

REMEDY: Repair of Heterozygous Mutations Independent of Exogenous Donor Template with High Efficiency
Meisam Naeimi Kararoudi, The Ohio State University, Columbus, OH

Speaking Slots Available

DAY-3	**NOVEMBER 16, 2024**	**SATURDAY**

Registrations

Panel Discussion

So the Data are Great, What Do We Need to Secure Venture Investment?

Moderator: **Gregory Fiore,** Board Member, Eterna Therapeutics

About Panel:
1. How do entrepreneurs judge the power and value of data they have generated on their compounds or with their platforms?
2. Ok, so everyone agrees the data are wonderful but how can we be sure investors aren't just going to ask for more?
3. What else do we need in addition to the data in order to secure a strong lead and syndicate?

Panelists:
Kaouthar Lbiati, Board Director, Hepion Pharmaceuticals
Wladimir Hogenhuis, CEO, Chimera Engineering
Anthony DiNatale, Associate, Agent Capital Inc
John Leonard, Chief Executive Officer, Leading Edge Bio, CA
Elizabeth Wood, Founder & CEO, Jura Bio Inc., MA

Coffee Break

TOPICS:
Gene Therapy for Blood Disorders
Commercialization Strategies and Market Analysis
Investment Trends in Gene Therapy Sector

TBA
Michael C. Rice, Head of Advanced Therapeutics, Lumanity

Assessing Biomarkers on Cell & Gene Therapy Clinical Trials: Considerations for Assay Development & Validation
Loren Blake, Study Director, FSP Solutions, Lab Connect

TBA
Susan J. Ward, Founder and Executive Director, cTAP-duchenne

TBA
Gbola Amusa, Chief Scientific Officer, Chardan

TBA
Kimberley Buytaert-Hoefen, Chief Scientific Officer, QPS LLC

TBA
Elizabeth Wood, Founder & CEO, Jura Bio Inc., MA

Networking Lunch

Gene Therapy Development Strategy for Future
Zhimei Du, Vice President Translational Research, Landmark Bio

Utilizing Point of Care Processing for Adoptive Cell Therapy
Gilad Beck, Director of Manufacturing, Orgenesis

Speaking Slots Available

**This is a draft program and the timings are subjected to change