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CELL & GENE THERAPY SUMMIT-2024

PROGRAM

THE GLOBAL
CELL & GENE THERAPY SUMMIT-2024

July 08-10, 2024 (Hybrid)

Boston Marriott Newton Hotel, Boston, MA

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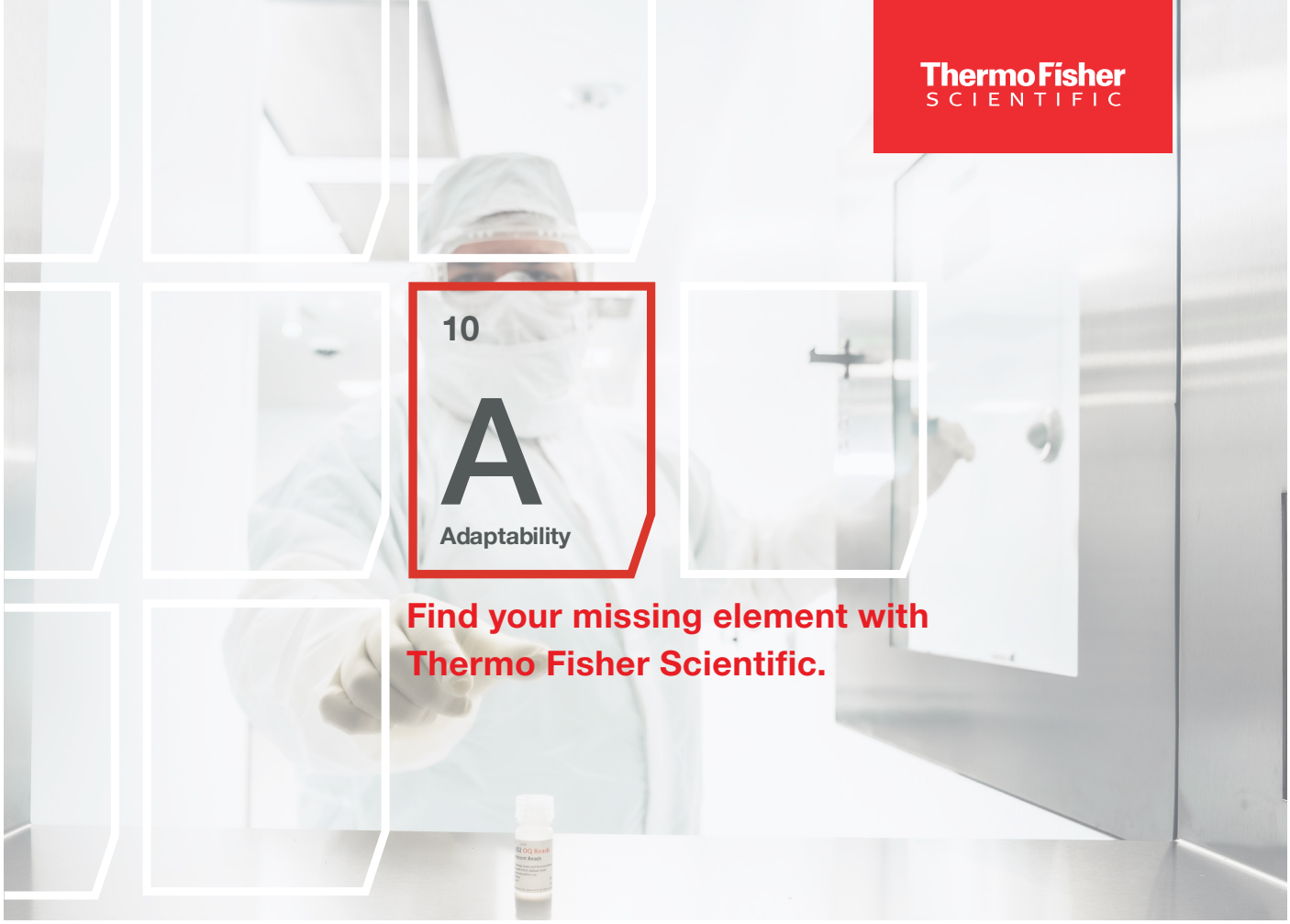

Find your missing element with Thermo Fisher Scientific.

Advanced therapy development requires adaptability

Due to their unique and complex nature, the development of novel advanced therapeutics often requires charting your own path and navigating through many unknown circumstances along the way. But this isn't a journey you have to go on alone.

At Thermo Fisher Scientific, we focus on understanding the unique needs of your advanced therapy so that we can innovate tailored solutions to meet them. Leveraging our extensive experience as a pharma services provider, combined with access to technical expertise and resources across our broader network, we provide you with the personalized support and adaptability needed to chart your own path with confidence.

We're here to help you every step of the way with industry-leading, end-to-end CDMO solutions, including:

- **Translational research services**
- **Development and manufacturing of viral vectors, cell therapies, and mRNA**
- **Cold and ultra-cold supply chain management and logistics**

Learn how we can support your advanced therapy's journey from development to delivery.

08:00 On-site Registrations

08:50 Introduction and Opening Remarks

Genome Engineering, Gene Regulation, Gene Editing Break Throughs & Therapeutic Approaches

Chair: Nidhi Kotecha, Gates Institute, CO

09:00 Keynote Talk: **Group II Intron Reverse Transcriptases: Drivers of Genome Evolution and Tool Boxes for Genome Engineering**
Alan M. Lambowitz, Professor of Molecular Biosciences, The University of Texas at Austin, TX

09:30 **Epigenetic Editing with CRISPR-GNDM: Novel Muscle-tropic AAV Vectors Deliver Promising Single-dose Treatment for LAMA2-CMD**
Tetsuya Yamagata, Chief Scientific Officer, Modalis Therapeutics Inc., MA

09:50 **Cis-regulatory Networks of Key Methylation Sites Describe Inter-patients Transcriptional Variation**
Asaf Hellman, Professor, The Hebrew University, Israel

10:10 Networking (Coffee) Break @ Salon D

Cell and Gene Therapies for Ocular Disorders

Chair: Demet (Dee) Sag, Genome2Life, MA

10:40 Keynote Talk: **The Future of Stem Cell Therapy in Ocular Disorders**
Ula V. Jurkunas, Co-director, Cornea Center of Excellence, Harvard University, MA

11:10 **'Mass-Scale' Cell Therapy for Corneal Endothelial Disease**
Greg Kunst, Chief Executive Officer, Aurion Biotech, WA

11:30 **AXV-101, a New Codon-optimised *BBS1* AAV9 Vector Halts Photoreceptor and Outer Nuclear Retinal Layer Organisation Degeneration in a Dose-dependent Manner**
Victor Hernandez, Chief Scientific Officer, Axovia Therapeutics Ltd, United Kingdom

Advancements in Cell and Stem Cell-based Therapies

Chair: Demet (Dee) Sag, Genome2Life, MA

11:50 Keynote Talk: **An Overview of Stem Cell Transplant: Current Scenario in Tissue Regeneration**
Anand Srivastava, Chairman & Co-founder, Global Institute of Stem Cell Therapy and Research (GIOSTAR), CA

12:20 Keynote Talk: **Innovative Approaches for Developing Pluripotent Stem Cell-based Therapies**
Erin Kimbrel, President, Astellas Institute for Regenerative Medicine, MA

12:50 **Pioneering Macrophage Cell Therapy in End Stage Liver Disease**
Amir Hefni, Chief Executive Officer, Resolution Therapeutics, United Kingdom

13:10 Lunch @ Salon D

Chairs: Lara Campana, Resolution Therapeutics, United Kingdom
Anand Srivastava, Global Institute of Stem Cell Therapy and Research (GIOSTAR), CA

14:10 **ProtoNK™ Cells from Pluripotent Stem Cells: Therapeutics for All Patients**
John Lu, President & Chief Executive Officer, HebeCell, MA

14:30 **Jump Start Allogenic Cell Therapies Using cGMP-Grade TARGATT Master iPSCs**
Ruby Tsai, CEO & Co-founder, Applied StemCell, CA

14:50 **Regenerative Medicine Using Conserved Mechanisms for Women's Health**
Demet (Dee) Sag, Founder, Genome2Life, MA

15:10 **Advancing Liver Disease Therapies: A Perfusable Vascularized Liver Organoid-on-a-chip Model**
Zhengpeng Wan (Jason), Research Scientist, Massachusetts Institute of Technology, MA

15:30 **Augmented Anti-tumour Potency via Functionalised Mesenchymal Stem Cells for Treatment of Peritoneal Carcinomatosis**
Jun Yung Woo, Post-doctoral Fellow, National University of Singapore, Singapore

15:50 Online: **Fine-tuning Immunity - *ex vivo* siRNA Treatment to Enhance Efficiency of Cell Therapies**
Anastasia Kremer, Researcher, University Hospital Tuebingen, Germany

16:10 Exhibitor Talk: **Assessing T Cell Potency Using Enrich TROVO System**
Qi Zhao, Founder & Chief Scientific Officer, Enrich Biosystems Inc., CT

16:30 Coffee Break @ Salon D

Gene Therapy: Methods, Strategies & Clinical Applications/ Precision Gene Therapy for Specific Disorders

Chair: Nidhi Kotecha, Gates Institute, CO

16:50 **Gene Therapy for Spinocerebellar Ataxia**
Luis Pereira de Almeida, President, CNC UC, Portugal

17:10 **Next Generation Synthetic DNA**
Elisa Cuevas, Head of NPD, 4basebio Discovery Ltd., United Kingdom

17:30 **Integrated Bioanalytical Approach to Characterize the Cellular Kinetics and Biodistribution to Support *ex vivo* and *in vivo* Gene Therapy**
Hiroshi Sugimoto, Associate Director, Takeda Development Center Americas Inc., MA

17:50 **Non-Lipid Polymer-based Nanoparticles - A Safe and Efficient Alternative for Gene Delivery**
Shira Orr, Chief Executive Officer & Co-founder, Envoya Inc., MA

18:10 Online: ***In vivo* Precision Gene Therapy for Beta-hemoglobinopathies**
Chang Li, Assistant Professor, Medical Genetics, University of Washington, WA

19:00 Onwards Social & Networking Dinner (Ticket Required)

08:40 Introduction and Day 2 Opening Remarks

Keynote Session

Chair: Alan M. Lambowitz, The University of Texas at Austin, TX

08:50 **RNA Immunotherapies for Cancer: The World's First CAR-mRNA-LNP Experience in Humans. *In vivo* Programming of Immune Cells Using mRNA-LNP Chimeric Antigen Receptors**
Jerome Chal, SVP, Head of CMC, Myeloid Therapeutics, MA

09:20 **MECP2. Gene Regulation and Therapeutic Approaches to Autism**
Rudolf Jaenisch, Professor of Biology, MIT, MA

09:50 **AI-driven Decision Making for Cell & Gene Therapy Production**
Irene Rombel, Chief Executive Officer & Co-founder, BioCurie, DE

10:20 Networking (Coffee) Break @ Salon D

CGT Advances in Cancer: Precision Therapies and Immunomodulation

Chair: Alan M. Lambowitz, The University of Texas at Austin, TX

10:50 **A New Approach to Tackle Cancer Therapies Using Programmable Cytotoxic Nucleases**
Michael Krohn, Co-Chief Executive Officer, Akribion Genomics, a BRAIN Biotech AG Spin-out, Germany

11:10 **New Immune Checkpoints: From Discoveries to Novel Drugs to Clinical Trials**
Xingxing Zang, Louis Goldstein Swan Chair in Cancer Research, Albert Einstein College of Medicine, NY

11:30 **Programming mRNA for Cancer Immunotherapy**
Jaspreet Khurana, Senior Director, mRNA Programming, Strand Therapeutics, MA

11:50 **OBX-115, an Interleukin 2 (IL2)-sparing Engineered Tumor-infiltrating Lymphocyte (TIL) Cell Therapy, in Patients (pts) with Immune Checkpoint Inhibitor (ICI)-resistant Unresectable or Metastatic Melanoma**
Parameswaran Hari, Chief Development Officer, Obsidian Therapeutics, MA

12:10 **Translational Development of a Novel CAR-T Cell Therapy, from Bench to Bedside**
Yan Luo, Assistant Professor, Mayo Clinic, FL

Advancing CGT Manufacturing: Optimization, Technologies, and Platforms

Chair: Larry Forman, CHO Plus, Inc., CA

12:30 **Opportunities and Value in Using Process Wide Digital Twins to Support Cell and Gene Therapy Production**
Joseph Pekny, Co-founder & Chief Executive Officer, Advanced Process Combinatorics Inc., IN

12:50 Sponsor Talk: **Leveraging a Flexible and Efficient Rapid Development Framework™ to Accelerate Development and Manufacturing of Cell and Gene Therapies**
Mindy Sadik, Director, Cell Therapy Sciences, Thermo Fisher Scientific, MA

13:10 Lunch @ Salon D

14:00 Online: **Resource Effective Development of CGT Processes with Hybrid Modeling and Transfer Learning**
Moritz von Stosch, Chief Innovation Officer, Datahow, Switzerland

14:20 **Isolating Full Capsids in AAV Production with IPP Technology**
Jason Criscione, Chief Scientific Officer, Enquyst Technologies, Inc., MA

14:40 **Development of HEK293 Cell Line for Optimal Production of Novel Capsids with Enhanced Brain Tropism**
Zeynep Guillemin, Senior Scientist, Voyager Therapeutics, MA

15:00 **An Automated Rapid CAR T Cell Manufacturing Process on a Single Platform**
Liping Yu, VP Applications, Applied Cells, CA

15:20 **Enabling Compliant Manufacturing of a iPSC-derived Therapies**
Lynne Frick, President & Chief Executive Officer, Cell X Technologies, OH

15:40 **Access to an ISO5 Cell Therapy Production Space Anywhere**
Alicia Henn, Chief Scientific Officer, BioSpherix, Ltd., NY

16:00 **Novel Opportunities to Advance Cell Therapy Cryopreservation and Logistics**
Stella Vnook, Chief Executive Officer, Likarda Inc., MO

16:20 Coffee Break @ Salon D

Optimizing AAV Manufacturing: Engineering, Processing, and Analysis

Chair: Zeynep Guillemin, Voyager Therapeutics, MA

16:40 **A Novel Cell Engineering Platform for High-yield AAV Production and Improved Manufacturability**
Larry Forman, Founder & Chief Executive Officer, CHO Plus, Inc., CA

17:00 **Lyophilization as an Effective Tool to Develop AAV8 Gene Therapy Products for Refrigerated Storage**
Nick Lai, Senior Scientist, Regeneron Pharmaceuticals, NY

17:20 **AAV Analytical Tools - Start to Finish**
Jesse Kay, Business Development Manager, PROGEN Biotech Inc., AR

17:40 Online: **Upstream and Downstream Process Development of AAV Production Platform**
Huiren Zhao, Principal Scientist, Amgen, CA

18:00 Poster Presentation and Networking Drinks @ Foyer

List of Poster Presentations

CGT-1 **Development of an Allogenic Cell Therapy for Parkinson's Disease Using Low-immunogenic Dopaminergic Progenitors Differentiated from Gene-edited Induced Pluripotent Stem Cells in an Experimental Animal Model**
Chia-Ling Hsieh, Development Center for Biotechnology, Taiwan

CGT-2 **Navigating Genetics of Dementia in the Indian Sub-continent**
Shrutee Jakhanwal, Strand Life Sciences, India
Gujarati Karan, Strand Life Sciences, India

CGT-3 **Aberrant Expression of TIM-4-L is a Common Feature of AML and a Potential Target for Engineered T Cell Therapy**
Brandon Cieniewicz, CERo Therapeutics Holdings, Inc., CA

CGT-4 **Spinal Muscular Atrophy and Pompe Disease Gene Therapy Clinical Trials in China**
Xiaodong Wang, GeneCradle Pharmaceutical Co., Ltd., China

CGT-5 **Influence of Cell Division on Plasmid Nuclear Entry and Expression**
Justin Sylvers, Duke University Durham, NC

CGT-6 **Non-lipid Polymeric Nanoparticle-encapsulated siRNA is Effective *in vitro* and *in vivo***
Trinayan Kashyap, Envoya Inc., MA

CGT-7 **Low Volume Wash, Formulation and Fill Strategies for T Cell Therapies**
Supriya Prakash, ElevateBio, MA

CGT-8 **Single Cell Fluorescence Lifetime Imaging for Subcellular Enzyme Activity**
Brock Harvey, University of Kentucky, KY

CGT-9 **Validation of an Impedance-based *in vitro* Potency Assay for Repeatability and Precision**
Danielle Califano, Axion BioSystems, NY

08:50 Introduction and Day 3 Opening Remarks

Regulatory Landscape and Commercialization Strategies

Chair: Nidhi Kotecha, Gates Institute, CO

09:00 **Keynote Talk: Preclinical Considerations for Cell and Gene Therapy Products**
Kimberley Buytaert-Hoefen, Executive Director & Global Head of Regulatory Services, QPS Holdings, LLC, CO

09:30 **Keynote Talk: The Unknown Unknowns - New Ways of Identifying and Leveraging Stakeholders in Order to Optimally Operationalize Innovation**
Rachel Salzman, Chief Executive Officer, Armatus Bio, OH

10:00 **Operational Complexity in Phase-I GMP Cell Therapy Manufacturing: A Compliance Perspective**
Nidhi Kotecha, Program Director, Regulatory Affairs, Gates Institute, CO

10:20 **Maximizing Regulatory and Patent Strategy for Cell and Gene Therapy Manufacturing Success**
Susan M. Faust, Patent Specialist, Hogan Lovells US LLP, DC
Sally Gu, Life Sciences Attorney, Hogan Lovells US LLP, DC

11:00 Panel Discussion - I
Navigating Regulatory Pathways: Strategies for Commercial Success

Nidhi Kotecha, Program Director, Regulatory Affairs, Gates Institute, CO
Rachel Salzman, Chief Executive Officer, Armatus Bio, OH
Susan M. Faust, Patent Specialist, Hogan Lovells US LLP, DC
Sally Gu, Life Sciences Attorney, Hogan Lovells US LLP, DC

11:40 Panel Discussion - II
Collaborative Approaches: Industry-Academia Partnerships for CGT

Xingxing Zang, Louis Goldstein Swan Chair in Cancer Research, Albert Einstein College of Medicine, NY
Michael Krohn, Co-Chief Executive Officer, Akribion Genomics, a BRAIN Biotech AG Spin-out, Germany
Kira Gillett, Program Manager, Foundation for the National Institutes of Health, MD
Luis Pereira de Almeida, President, CNC UC, Portugal

12:30 Lunch & Departure @ Salon D

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- Next Generation Sequencing
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CRISPR QC's CRISPR Analytics Platform provides comprehensive measurements and analysis around CRISPR activity, enabling scientists and researchers to optimize gene editing outcomes and ensure the highest levels of quality control. The platform is built on the company's proprietary CRISPR-Chip technology, which allows for direct, real-time measurement of CRISPR activity.

Envoya is a drug delivery company focused on developing a non-lipid polymer delivery platform for gene therapy. Our platform aims to harness the power of natural and synthetic polymers to overcome the challenges traditional gene therapy delivery systems face and unlock new possibilities for treating a wide range of genetic disorders.

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- *Non-immunogenicity*
- *High Transfection Efficacy*
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ChemoMetec is a leading manufacturer of cell counting and analysis equipment worldwide. ChemoMetec specialize in developing and producing high-precision instrumentation, delivering consistent data for thousands of customers across biotech, pharma and academic segments.

ZERO GRAVITY is committed to produce effective system of LED light therapy (light emitting diodes) for facial skin rejuvenation in the industry. Backed by medical science but specifically modified to allow for aesthetic-only treatments, the sophisticated science behind infrared light therapy is supported by many leading board-certified dermatologists and plastic surgeons across the globe.

We wish to see you at

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Boston, MA

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