

The Global Cell & Gene Therapy Summit

July 08-10, 2024 (Hybrid)
Boston Marriott Newton Hotel, MA

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Website: <https://cgtsummit.com/>

Welcome to CGT Summit 2024

We are thrilled to extend a warm welcome to all participants of the CGT Summit 2024. This summit will bring together exceptional experts from academia and industry, fostering discussions on diverse topics of cell and gene therapy. CGT Summit's comprehensive agenda will feature talks on topics ranging from genome engineering, gene regulation, and viral vector purification to precision gene therapy, cell therapy innovations, and immunotherapy advances.

CGT sessions also list talks on AI-driven decision-making, efficient process development, compliant manufacturing of iPSC-derived therapies, AAV production optimization, closed and automated manufacturing processes with regulatory considerations, public-private partnerships, clinical trial strategies, and the pivotal journey of scaling cell therapies from trials to commercialization.

With a thematic focus on "From Basic Research to CGT Products: Advancing Cell and Gene Therapies through Innovation, Technology, and Collaboration," the summit aims to have meaningful discussions and impactful networking sessions. We look forward to dynamic discussions, insightful presentations, and collaborative endeavors that will undoubtedly drive progress in the realm of cell and gene therapies.

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EXPERTS @ CGT SUMMIT 2024



Luis Pereira de Almeida
President
CNC UC, Portugal



Rachel Salzman
CEO
Armatus Bio, OH



Irene Rombel
CEO & Co-founder
BioCurie, DE



Elisa Cuevas
Head of NPD
4basebio Discovery Ltd,
UK



Joseph Pekny
Co-Founder/CEO
Advanced Process
Combinatorics Inc., IN



Amir Hefni
CEO
Resolution
Therapeutics,
UK



Jerome Chal
SVP, Head of CMC
Myeloid Therapeutics,
MA



Hiroshi Sugimoto
Associate Director
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MA



Lynne Frick
President & CEO
Cell X
Technologies,
OH



Kira Gillett
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Jason Criscione
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Larry Forman
Founder and CEO
CHO Plus, Inc.,
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Xingxing Zang
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Mindy Sadik
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Chief Innovation Officer
Datahow, Switzerland



John Lu
President & CEO
HebeCell, MA



Rudolf Jaenisch
Professor
MIT, MA



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



Ula V. Jurkunas
Co-director, Cornea
Center of Excellence
Harvard
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Chang Li
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Nidhi Kotecha
Program Director,
Regulatory Affairs,
Gates Institute,
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Alicia D. Henn
CSO
BioSpherix, Ltd., NY



Anand Srivastava
Chairman &
Cofounder
GIOSTAR, CA



Liping Yu
VP, Application
Applied Cells,
CA



Ruby Tsai
CEO & Co-Founder
Applied StemCell, CA



Asaf Hellman
Professor
The Hebrew
University, Israel



Susan M. Faust
Patent Specialist
Hogan Lovells
US LLP, DC



Kimberley Buytaert-Hoefen
Executive Director & Global
Head of Regulatory Affairs
QPS LLC, DE



Sally Gu
Senior Associate
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Hui ren Zhao
Principal Scientist
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Program Outline

Day 1, July 08, 2024

Opening Remarks

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

Networking (Coffee) Break

Cell and Gene Therapies for Ocular Disorders

Advancements in Cell and Stem Cell-based Therapies

Lunch

Advancements in Cell and Stem Cell-based Therapies *continues...*

Coffee Break

Gene Therapy: Methods, Strategies & Clinical Applications/ Precision

Gene Therapy for Specific Disorders

***Social & Networking Dinner (Ticket Required)**

Day 2, July 09, 2024

Keynote Session - Day 2

Networking (Coffee) Break

CGT Advances in Cancer: Precision Therapies and Immunomodulation

Advancing CGT Manufacturing: Optimization, Technologies, and Platforms

Lunch

Advancing CGT Manufacturing: Optimization, Technologies, and Platforms *continues...*

Coffee Break

Optimizing AAV Manufacturing: Engineering, Processing, and Analysis

Poster Presentation and Networking Drinks

Day 3, July 10, 2024

Regulatory Landscape and Commercialization Strategies

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

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

Closing Remarks

Lunch & Departure

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. Jurkunus, Co-director, Cornea Center of Excellence, Harvard University, MA

11:10 'Mass-Scale' Cell Therapy for Corneal Endothelial Disease

Greg Kunst, CEO, 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, UK

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 & Cofounder, 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, CEO, 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 ProtoNKT^M Cells from Pluripotent Stem Cells: Therapeutics for All Patients
John Lu, President and CEO, 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, Cambridge, 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 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, CSO, 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, CEO & 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 Worlds 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, *CEO and 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-CEO, 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 & CEO, 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 Guillemmin, 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 & CEO, Cell X Technologies, OH

15:40 Access to an ISO5 Cell Therapy Production Space Anywhere
Alicia Henn, CSO, BioSpherix, Ltd., NY

16:00 Novel Opportunities to Advance Cell Therapy Cryopreservation and Logistics
Stella Vnook, CEO, Likarda Inc., MO

16:20 **Coffee Break** @ Salon D

Optimizing AAV Manufacturing: Engineering, Processing, and Analysis

Chair: *Zeynep Guillemmin, Voyager Therapeutics, MA*

16:40 A Novel Cell Engineering Platform for High-yield AAV Production and Improved Manufacturability
Larry Forman, Founder and CEO at 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
Hui ren 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, Beijing 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 and 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, *CEO, Armatius 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, *Senior Manager, Quality Assurance, Gates Biomanufacturing Facility, CO*
Rachel Salzman, *CEO, Armatius 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-CEO, 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

Participants From



DATAHOW

PROGEN

AMGEN



FNIH

Foundation for the
National Institutes of Health



Applied Cells



TEXAS

The University of Texas at Austin

REGENERON



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Participants From



CellX
TECHNOLOGIES

CHO+Plus



Roche Roche Group

W
UNIVERSITY of
WASHINGTON

 **astellas**
Institute for
Regenerative Medicine



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MEDICAL SCHOOL

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THE HEBREW UNIVERSITY OF JERUSALEM

 **ZS**

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Universitätsklinikum
Tübingen

 **OBSIDIAN**
THERAPEUTICS

Intellia
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voyager
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AND CELL BIOLOGY
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 **MassBiologics**
Medicine for Better Lives

**Hogan
Lovells**

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Collaborate with CGT Summit 2024

Calling all visionary startup founders, R&D experts, researchers, and industry leaders driving innovation in advanced manufacturing platforms, bioreactor capabilities, and other cell and gene therapy development aspects: Collaborate with CGT Summit 2024. Engage in in-depth discussions and tap into invaluable market intelligence. The summit offers abundant collaboration opportunities, industry insights, and a global perspective, providing an unparalleled platform to unlock key partnerships and explore transformative collaborations.

Collaboration Prospects

Explore transformative partnerships for innovative cell and gene therapies

Industry Insights

Gain crucial industry insights to propel your organization's success

Post-Conference Resources

Get Exclusive post-conference resources for continued learning and reference after the event concludes

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