ADVANCING GENE THERAPY

28-30 MARCH 2022
BOSTON, MA

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BIA Separations, recently acquired by Sartorius, develops, and manufactures market leading CIM® monolithic chromatographic columns for purification and analysis of large biomolecules, such as viruses, plasmids, and mRNA, which are applied in cell and gene therapies. BIA’s technology for manufacturing-scale purification is already used in production of the first commercialized advanced therapeutics; BIA also has a keen presence with novel drug candidates in the clinical pipeline. Expecting continued strong double-digit sales growth over the next few years, BIA earned sales revenue approx. 25 million Euros in 2020, contributing to Sartorius Bioprocess Solutions, part of the Sartorius Group.

NOF CORPORATION is the leading commercial supplier of GMP grade activated PEGs, lipids and ultrapure Polysorbates 80 for drug delivery products through R&D to commercial scale. We provide a wide range of lipids (ionizable, PEG, and phospho) for lipid nanoparticle formulations (LNP). Our LNP platform, containing our ionizable lipid (COATSOME® SS Series), has excellent endosomal escape ability and provides high gene expression with low toxicity. It can apply to gene delivery, gene editing, and RNA vaccine application under this platform.
Meet the industry forerunners at BSC’s inaugural Advancing Gene Therapy 2021 to gain insights into their strategies and program development. The Summit will highlight the challenging field of gene therapeutic approaches. Advances in development and manufacturing will be presented, as well as quality control methods and regulatory aspects: The focus is on the path to safe and beneficial advanced therapies for the patient.

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08:30 - 08:35  Introduction  **Michael Fossel**, President and Founder, Telocyte

**Chair:**  **Michael Fossel**, President and Founder, Telocyte

### Keynotes

08:35 - 09:15 - **Christian Mueller**, Global Head of Genomic Medicine, Sanofi Genzyme  
**Title:** Gene Therapy with Next Generation AAV Capsids and Non-Viral Vectors

09:15 - 09:55 - **Michael Fossel**, President and Founder, Telocyte  
**Title:** Understanding Aging, Curing Disease

09:55 - 10:40  Networking and Refreshments Break  
Sponsored by Houston Gene Therapeutics

### Session: Gene Therapy for Neurological Disorders

10:40 - 11:05 - **Shane Hegarty**, CSO & Co-Founder, AXONIS Therapeutics, Inc.  
**Title:** In vivo Phenotypic Screens to Identify New Gene Therapies for Neurological Disorders

### General Session

11:05 - 11:45 - **Syed Reza**, Drug Delivery Consultant, NOF Corporation  
**Title:** COATSOME SS-Lipids as Novel, Biodegradable, Ionizable Lipids for Nucleic Acid Delivery and Next Generation Vaccines

11:45 - 12:25 - **Sarah Moore**, Head of Gene Therapy Discovery, Touchlight Genetics Ltd  
**Title:** Advancing Genetic Medicines with Enzymatically Amplified dbDNA

12:25 - 13:25  Lunch

### Session: Gene Therapy for Rare Diseases

13:25 - 13:50 - **Kristin Heller**, Plasmid Core Manager, Andelyn Biosciences  
Co-Presenter: Jonathan Rush, Head of Supply Chain, PMO and Operational Excellence, Andelyn Biosciences  
**Title:** Supply Chain Stability vs. Instability in 2022 and beyond.

13:50 - 14:15 - **Dan Wang**, Assistant Professor, Horae Gene Therapy Center | RNA Therapeutics Institute, University of Massachusetts Chan Medical School  
**Title:** Targeting Common Causes in Rare Diseases
**Keynote**

14:15 - 14:55 - Jeff Galvin, CEO & Founder, American Gene Technologies  
**Title:** Gene and Cell Therapy Revolution: The Future of Pharmaceutical

**Session: Gene Therapy for Cancer & Immunology**

14:55 - 15:20 - Anthony Johnson, Chief Executive Officer/President, Kodikaz  
**Title:** ZipCodes: The Next Generation Gene Therapy in Oncology

15:20 - 15:45 - George Tetz, Chief Executive Officer, CLS Therapeutics  
**Title:** First-In-Class anticancer AAV-based (LK03) Gene Therapy Based on The Destruction of Neutrophil Extracellular Traps with Transgene Expression of DNase I in Liver.

15:45 - 16:10 - Khalid Shah, Vice Chair of Research, Director, Center for Stem Cell and Translational Immunotherapy, BWH, Harvard Medical School  
**Title:** Cell Based Immune Therapies for Solid Tumours

16:10 - 16:30  
**Networking and Refreshment Break**

**Session: Gene Therapy for Neurological Disorders**

16:30 - 16:55 - Joerg Ahlgrimm, President and COO, Center for Breakthrough Medicines  
**Title:** How a Comprehensive Outsourcing Solution Accelerates Speed to Milestone

**Posters**  

17:00 - 17:30

P1: Jake McAndrew, Bioprocessing Product Manager, Distek, Inc.  
**Title:** A Demonstration of the Effectiveness of Response Surface Design of Experiments (DoE) Modeling for Upstream Bioprocess Optimization

P2: Heather Griffith, Senior Bioprocess Specialist, Pall Biotech  
**Title:** Clarification with Pall Seitz® Depth Filters for Adherent and Suspension Adeno-Associated Virus Culture

P3: Timotej Zvanut, Scientist at Analytical Development, BIA Separations  
**Title:** Empty/Full Capsids Separation Columns for Determination of DNA-related Impurities

19:00 -  
**Networking Dinner**
Session: Analytical Development and Manufacturing

08:30 - 09:30  Panel Discussion: Gene Therapy Analytical Development and Manufacturing

Topics to be Discussed:
- Analytical Analysis, Key Regulatory Considerations, and Manufacturing
- Up Scaling and Regulatory Considerations on the Way to Commercialization
- Ensuring Scalability and Efficient Timelines in Manufacturing While Still Maintaining a Reasonable Cost

Moderator
Sean Smith
Sr. Director of Quality Assurance and Validation, Homology Medicines

Panellist 1
Ivana Petrović Koshmak
Head of Upstream Process Development, Department for Process Development, BIA Separations

Panellist 2
Kim Raineri
Chief Manufacturing and Technology Officer, AVROBIO

Session: Vector Products & Innovative Delivery Platforms

Chair: Syed Reza, Drug Delivery Consultant, NOF Corporation

Keynote

09:30 - 10:10 - Syed Reza, Drug Delivery Consultant, NOF Corporation
Title: COATSOME SS-Lipids as novel, biodegradable, ionizable Lipids for mRNA Therapeutics

10:10 - 10:35 - Joseph Rininger, Director, Cell and Gene Therapy, Latham Biopharm Group
Title: Analysis of Viral Vector Demand versus CDMO Capacity to Meet that Demand

10:35 - 10:50  Networking and Refreshment Break

10:50 – 11:15 - Ivana Petrović Koshmak, Head of Upstream Process Development, Department for Process Development, BIA Separations (A Sartorius Company)
Title: AAV Manufacturing and In-Process Controls

11:15 - 11:40 - Iker Badiola, Professor, University of the Basque Country
Title: Sorbitan Ester based Nano system for MiRNA Delivery as Antiangiogenic Therapy
11:40 - 12:05 - Arun Srivastava, George H. Kitzman Professor of Genetics, Chief, Division of Cellular and Molecular Therapy, Powell Gene Therapy Center, University of Florida College of Medicine
Title: Development of NextGen, GenX, and Opt AAV Vectors for Human Gene Therapy

12:05 - 12:30 - Richard Heller, GProfessor, Department of Medical Engineering, University of South Florida
Title: Gene Electrottransfer of Plasmid DNA as an Effective Delivery Approach for Multiple Therapeutic Applications

12:30 - 13:30  Lunch

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**Session: Commercialization Strategies, Business Models and Market Access**

**Chairs:** Rachel Legmann, Director of Technology, Gene Therapy, Repligen Corporation  
Kevin MacDonald, Associate, Wolf Greenfield & Sacks, P.C.

**Keynote**

13:30 - 14:10 - Clark Pan, Vice President, Gene Therapy, Pfizer
Title: Building an Industry-Leading Gene Therapy Pipeline

14:10 - 14:35 - Angela Columbano, Head Business Development and Partnership, Genethon
Title: Genethon: Building a Strong and Unique Pipeline of Gene Therapies for Neuromuscular Diseases

14:35 - 15:00 - Rachel Legmann, Director of Technology, Gene Therapy, Repligen Corporation
Title: Intensification of Viral Vector Manufacturing Upstream and Downstream Processes

15:00 - 15:25 - Jean-Simon Diallo, CEO & Scientific Founder, Virica Biotech
Title: Increasing Gene Therapy Vector Production using Viral Sensitizer Molecules

15:25 - 15:50 - Markus Haindl, Head Gene Therapy Technical Development, Roche
Title: Considerations Towards Development & Industrialization of In Vivo Gene Therapies

15:50 - 16:10  Break
16:10 - 16:35 - Susan G. L. Glovsky, Principal, Hamilton, Brook, Smith & Reynolds, P.C.
Title: Erecting and Tearing Down Patent Barriers to Market Access

16:35 - 17:00 - Kevin MacDonald, Associate, Wolf Greenfield & Sacks, P.C.
Title: Becoming 'Diligence-ready' Implementation of IP Strategies for early-stage gene therapy companies

17:00 - 17:25 - Jeff Gilmore, Director, Chief Executive Officer, cGMPnow
Title: Speed to Market: Accelerating GMP Readiness of an ATMP Manufacturing Facility

17:25 - 17:50 - Audrey Greenberg, Co-founder, Executive Director and Board Member, Center for Breakthrough Medicines
Title: Creating a Successful Bio Innovation Hub

17:50 - 18:15 - Emily Moran, Senior Director, Viral Vector Manufacturing, Center for Breakthrough Medicines
Title: Commercialization Strategies and the Industrialization of Academic Processes from Development to Manufacturing
### Session: Gene Editing & Crispr

08:30 - 09:30  **Panel Discussion: Integrated Solutions for Cell and Gene Therapy**

**Description:** As cell and gene therapies continue to advance, it’s essential to have innovative solutions to address the myriad of complexities that present throughout the drug discovery, development, and manufacturing process. In this panel, parties will discuss various impediments and barriers faced as well as why integrated technological solutions are necessary.

**Topics to be Discussed:**

- The potential of cell and gene therapies and their impact on the future of drug development
- Complexities/barriers/challenges that present in cell and gene therapy advancements
- Integrative solutions to innovate, develop, and accelerate life-transforming therapies
- Effective coordination to drive precision, efficiencies, and quality
- Delivering on the needs – manufacturing for demand, scale, and beyond

| Moderator | Kareem Reda, MBA  
| Chief Business Officer | ElevateBio |

| Panellist 1 | Clare Murray, PhD, MBA, SVP  
| Corporate Development & Operations, Life Edit Therapeutics |

| Panellist 2 | Akira Matsuno  
| Co-founder, President and CFO, Tune Therapeutics |

| Panellist 3 | Omar Abudayyeh  
| Independent MIT McGovern Fellow at the McGovern Institute for Brain Research, MIT |

| Panellist 4 | Jonathan Gootenberg, PhD  
| MIT McGovern Fellow at the McGovern Institute for Brain Research, MIT |

09:30 - 09:50  **Break**

09:50 - 10:15  **Devyn M. Smith**, Chief Executive Officer, Arbor Biotechnologies, Inc.

**Title:** Using Next-Generation Genome Editors Tailored for Patient Diseases

### Session: Cell Therapy & CAR-T

**Chair:**  
Farideh Bischoff, CEO, CSO and Board Director, FreMon Scientific

**Keynote**

10:15 - 10:55  **Will Chou**, Chief Executive Officer, Aruvant

**Title:** Differentiated Cell Therapy approach for Sickle Cell Disease
10:55 - 11:20 - Dan Shelly, Vice President Business Development and Alliances, Prescient Therapeutics

**Title:** OmniCAR Development for a Universal CAR-T Therapy

11:20 - 11:45 - Farideh Bischoff, CEO, CSO and Board Director, FreMon Scientific

**Title:** Efficacy of a Rapid Dry Precision Thawing Platform for Point-of-Care Cellular Therapies

11:45 - 12:10 - Tamer Mohamed, Chief Executive Officer, Aspect Biosystems

**Title:** Bioprinted Cell Therapies as Medicines of the Future

12:10 - 13:00  Lunch

### Session: Toxicology Studies

13:00 - 13:25 - Michele Stone, Vice President Translational Development, Kriya Therapeutics

**Title:** Challenges in Nonclinical Pharmacology and Toxicology Gene Therapy Studies

13:25 - 13:50 - Daniel Kavanagh, Senior Scientific Advisor, Gene Therapy, WCG IRB

**Title:** Ethical and Biosafety Review of Gene Therapy Clinical Trials: Special Consideration for Oversight by IRBs and IBCs.

### Session: Gene Therapy for Ocular Diseases

13:50 - 14:15 - Magali Taiel, Chief Medical Officer, GenSight Biologics

**Title:** Lumevoq Gene Therapy in Leber Hereditary Optic Neuropathy (LHON) Subjects

14:15 - 14:40 - Samarendra Mohanty, President & Chief Scientific Officer, Nano scope Therapeutics Inc.

**Title:** Pioneering New Wave of Optogenetic Therapeutics for Vision Restoration

### General Session

14:40 - 15:05 - Qizhen Shi, Blood Research Institute, Versiti Wisconsin, Milwaukee, WI, USA

**Title:** Platelet-specific Gene Therapy for Hemophilia

15:05 – 15:15  Closing Remarks
WHO WILL BE THERE?
DQUAD 2022

Drug Discovery, Delivery and Development 2022
19-22, September, 2022
San Francisco, CA

DQUAD 2022 enables you to meet with pharma and biotech Pioneers at the forefront of research, this conference will highlight the latest scientific breakthroughs, exploring innovative technologies and approaches that can be used to overcome discovery and development challenges, and bring together industry leaders who will discuss future opportunities for novel therapeutic discovery.

For more details: https://biogatesc.com/events/DQUAD2022/
On May 22, please join us at our Rare Disease Therapeutic Alliance 2023, which will feature 1on1 meetings with the select group of specialty pharma and biotech companies focused on developing therapies for some of the rare and orphan diseases. Investors will have the opportunity to meet with management teams to discuss in detail key therapeutic programs in development and recent corporate updates.

For more details: https://biogatesc.com/events/rarediseases/
THANK YOU

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