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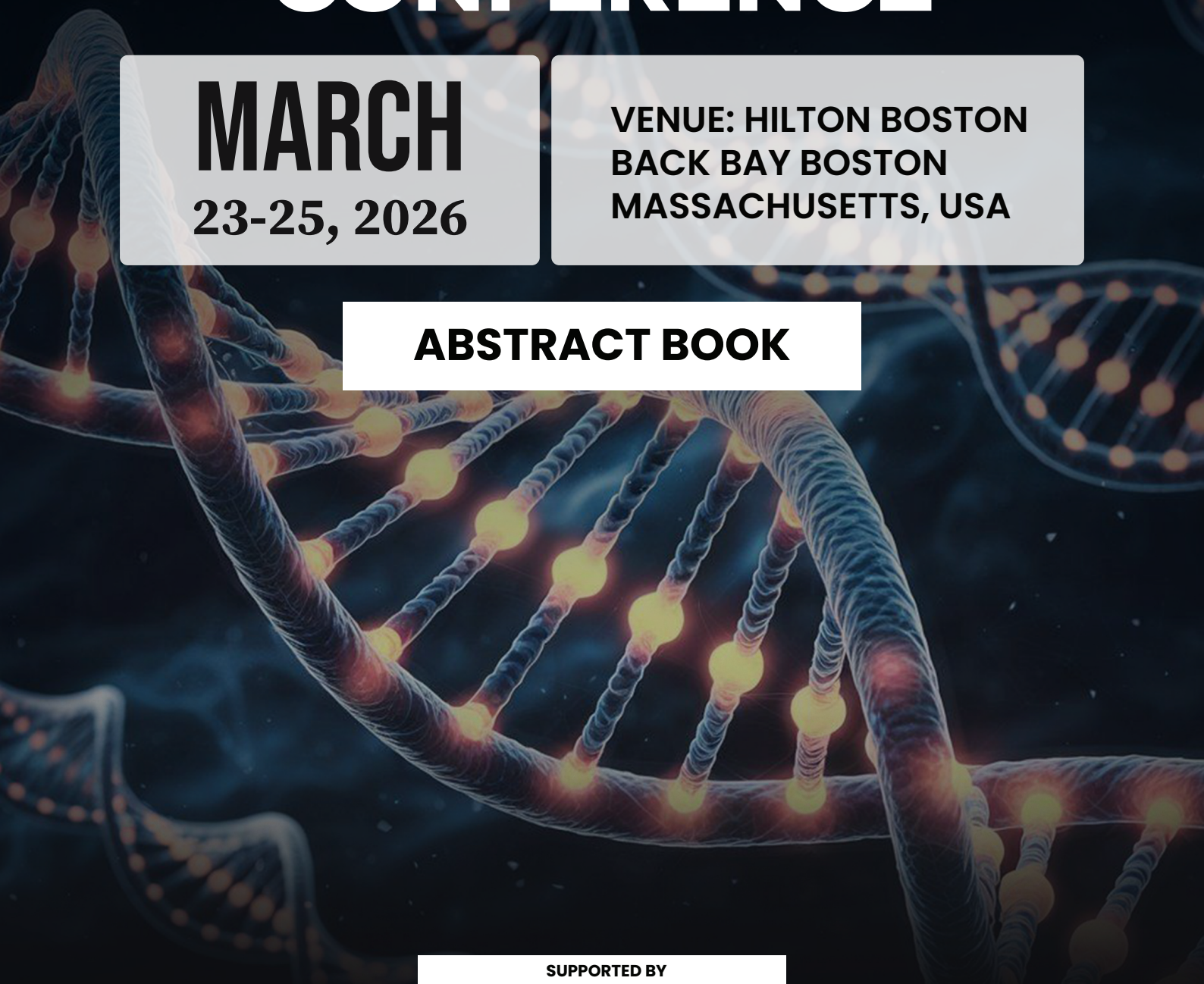
ABSTRACT BOOK



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Expanding Gene Editing Beyond the Liver

Devyn Smith, Arbor Biotechnologies, USA

Development of Capsid- and Genome-Modified NextGen AAV Vectors; Nuclease-Free Gene Editing With AAV-B19 Hybrid and Chimeric Vectors

Arun Srivastava, University of Florida College of Medicine, USA

Mitigating Immune Responses to AAV Vector-Mediated Gene Transfer

Hildegund Ertl, The Wistar Institute, USA

Genome Integrity: Screening, Characterization, and Optimization

Jeremy Shelley Director of Business Development, Form Bio, USA

Abstract:

In gene therapy development, genome integrity directly influences program cost, speed, and clinical outcomes. Sequence-driven instability can lead to incomplete or heterogeneous vector genomes, reduced manufacturing yields, inconsistent potency, failed scale-up, and toxicity signals. At late preclinical development, tech transfer, GMP manufacturing, or worst - in the clinical stages - remediation often requires extensive re-engineering and leads to manufacturing delays or regulatory rejection, driving significant unplanned cost and eroding development timelines, ultimately impacting the bottom line.

Predictive genome integrity screening enables developers to identify and mitigate sequence-related manufacturing risks early, when design changes are fastest and least disruptive. By evaluating genome stability and manufacturability in silico prior to experimental execution, teams can prioritize candidates with higher likelihood of scalable, repeatable production. Furthermore, characterization using long-read sequencing (LRS) at multiple stages of development offers unprecedented genomic resolution into the capsid contents and de-risks each development step to ensure the best outcome. Finally, transgene optimization with FORMsightAI leads to programs showing, on average, ~2.3x decrease in contaminants, 1.4x increase in protein expression, 2.3x increase in genome integrity and 1.7x increase in yield.

Embedding predictive genome integrity screening, LRS characterization, and FORMsightAI optimization into gene therapy R&D workflows supports more informed portfolio decisions, de-risks CDMO engagement, and preserves capital by preventing avoidable redesigns. As the industry moves

toward larger trials and commercial manufacturing, design for manufacturing becomes a strategic lever—not just a technical consideration—for controlling development risk, accelerating time to clinic, and maximizing return on investment across gene therapy pipelines.

Biography:

Jeremy Shelley is the Director of Business Development for Form Bio, Inc., leading business development partnering with Pharma and Biotech customers throughout the USA and Europe. Shelley holds a bachelor's in business administration from the University of Alabama and has

spent the last decade providing technology and consulting solutions to the life science community. As Director of Business Development, Jeremy partners with customers throughout Cell and Gene Therapy to define challenges within their organization, share and gather market trends, and provide solutions. Jeremy is located in the Greater Boston, Massachusetts, USA.

Gene Delivery Technologies

Molly S. McGlaughlin

Chief Executive Officer, Revolution Biomanufacturing Inc. (Previously known as Kudo Biotechnology)

Biography:

Ms McGlaughlin has over 29 years of experience in the Biotechnology industry in developing Biologic manufacturing business units, operational excellence, licensing and partnering technology, and fundraising. She has a solid track record of P&L performance and business strategy. Ms McGlaughlin has held positions on the management team of List Bio, Eirgenix, Natrix Separations, BioVectra, Selexys S.A., Cytovance Biologics, Avecia Biotechnology, Patheon, Mallinckrodt, and, most recently, as Chief Operating Officer of Vigene Bioscience, a Gene and Cell therapy Contract Development and Manufacturing Organization (CDMO). Currently, she is the Chief Executive Officer of Revolution Biomanufacturing, an mRNA full-service CDMO, responsible for fundraising, Corporate Development, operations, regulatory compliance, and all aspects of the company's day-to-day management.

Molly holds a BS in Biology from Norwich University, Military College of Vermont, a master's in organizational performance Improvement from the University of Massachusetts, Boston, a master's in Strategic Studies from the U.S. Army War College, and is ABD in the School of Business and Technology at Capella University, where her doctoral studies focus on M&A in the Biotechnology Industry. Molly has recently retired from the U.S. Army Reserves, where she held the rank of Colonel and commanded a Brigade twice.

New Technologies to Advance Manufacture, Delivery and Quality in AAV Gene Therapy

Daozhan Yu, USA

Abstract:

AAV gene therapy is hindered by manufacture difficulty, low delivery efficiency, toxicity and high-cost issues. At AAVnerGene, we have developed new technologies to solve these bottleneck problems. AAVone is an innovative one plasmid AAV production system. It produces 5X1015 GC/L of cell culture, which is about 10 times higher than current AAV production system. The full AAV particle ratio can reach >70% at harvest. The new system dramatically increases the productivity and quality, and reduces the cost, solving the manufacturing problem. It has been licensed to different companies for manufacturing of clinical AAV products. ATHENA is a AAV capsid engineering and screening platform, which can generate new AAVs targeting different tissues with higher delivery efficiency and specificity. For example, we have developed a new capsid, AAV-ShD, which can pass blood brain barrier and infect brain cells with hundreds of folds higher efficiency than AAV9. We are using the capsid to develop some new gene therapy pipelines for Alzheimer's disease, brain tumor and depression. These pipelines use much less dosage, and significantly reduce the toxicity and cost. AAV-Q is a new TCID50 for measuring AAV infectious activity. This assay is supersensitive and can reliably get accurate data to monitor AAV activity and direct the dosage.

These technologies solve the key problems in AAV gene therapy, and support efficient, safe and cost-effective gene therapy drug development.

Biography:

Dr. Daozhan Yu is the CEO of AAVnerGene, a biotech company located at Rockville, Maryland, focusing on new technology development to increase AAV gene therapy efficiency.

He graduated at University of Maryland at Baltimore in Molecular Medicine. He had been worked on different diseases including Diabetes, Obesity, Cardiovascular disease, Neuronal disease and cancers for 20 years. In the recent 10 years, he has been focusing on AAV gene therapy. In 2019, he co-founded the AAVnerGene Inc, which is now a leader in AAV technologies.

Gene editing therapy development: platform design and manufacturing readiness

Kok-Seong Lim

Abstract :

CRISPR therapies offer unprecedented potential for treating rare genetic diseases, yet their development remains constrained by high costs, manufacturing complexity and regulatory unpredictability. Platform technologies such as standardized systems for delivery, manufacturing and analytics are increasingly recognized as critical pathways to overcome these barriers. Operationalizing these platforms requires integrated strategy spanning manufacturing readiness, early regulatory engagement and clinical trial design. This presentation explores the strategic and technical aspects of building scalable gene editing platforms and discusses emerging regulatory pathways along with key lessons learned from recent programs.

Biography:

Kok-Seong Lim, BPharm, PhD, is a pharmaceutical leader with over 20 years of experience in biological research and development, specializing in analytical sciences, quality control and CMC strategy. He has held management roles at Metagenomi, Aura Biosciences, Editas Medicine and Thermo Fisher Scientific, where he supported the development of more than 10 gene therapies across rare disease and oncology. He serves as Vice Chair of the Regulatory Affairs Professional Society San Francisco Chapter and as a member of the U.S. Pharmacopeia Biologics Cell and Gene Therapy Expert Committee. He earned his PhD in Biochemistry from the National University of Singapore.

Isaralgagene Civaparvovec (ST-920) Shows Positive Mean Annualized eGFR Slope in Adults With Fabry Disease: Topline Results From the Registrational Phase 1/2 STAAR Gene Therapy Study and Long-Term Follow-Up Study

Mitra Tavakkoli, Sangamo Therapeutics ,
USA

Gene Therapy for X-linked Retinitis Pigmentosa

Daniel C. Chung, Beacon Therapeutics, USA

Cell and gene therapies, rare diseases and early phase units: A community site story

*Anita Mardian, PharmD1, *Chris A. Learn, PhD, MBA2*

Biography:

The enrollment of patients in Phase I Cell and Gene Therapy (CGT) and Rare Disease (RD) trials predominantly occur in academic centers due to the required expertise, safety and regulatory framework. While these centers remain essential, this presents a unique opportunity for a non-academic community-focused investigator site to support the growing numbers of Early Phase I CGT trials. In this alternative model, operational flexibility and expedited site activation complement the sponsor's overall development plan, as well as local access to patient queues that may not matriculate at larger research hospitals.

Objective:

Leveraging existing expertise in Phase I trials and in close collaboration with a pharmaceutical sponsor and community practitioner, Parexel's Early Phase Clinical Unit initiated a comprehensive Gap Analysis to expand its capability to enroll patients in Phase I CGT and RD trial in our community setting.

Methods:

The Gap Analysis, in alignment with Foundation for the Accreditation of Cellular Therapy (FACT) standard, was performed by an external party and a structured report was issued. The report identified a need for CGT specific Operating Procedures to address patient safety, product handling and storage, chain of custody, training, and quality standards.

Results:

The Gap Analysis is active and on track to be completed prior to first patient enrolled.

Conclusion:

With the recommended FACT standards in place, a non-academic community focused investigator site may support enrollment of patients in Phase I CGT and RD trials, increasing enrollments and patient access in ways not previously common to some intent to treat populations.

Biography:

Dr. Mardian is Head of Parexel's Early Phase Clinical Unit in Los Angeles and has over 20 years of experience in clinical drug development and patient care. Within this unique development setting, Dr. Mardian and her team have enrolled thousands of healthy volunteers and patients alike.

Bringing 25 years of clinical trial execution and team management experience, Dr. Learn is committed, enthusiastic, and passionate about translational science. With interests in strategic development of novel therapeutics, he is a published author and has been part of five marketed product approvals to date.

Targeting DNA Methyltransferase to Restore Vision after Traumatic Optic Nerve

Wai Lydia Tai

Abstract:

Optic nerve injury and neurodegenerative diseases in the eye cause irreversible blindness in adult mammals due to the limited regenerative capacity of the central nervous system. Emerging evidence, including our own findings, implicates epigenetic regulation—particularly DNA methylation—as a critical determinant of neuronal regenerative potential. Through combined small-molecule and genetic screening of epigenetic regulators, we identified DNA methyltransferase 3A (DNMT3A) as a potent intrinsic inhibitor of axon regeneration in both mouse and human retinal explant models. Targeted suppression of DNMT3A in retinal ganglion cells (RGCs), achieved via genetic ablation or shRNA-based gene therapy, induced robust, longdistance axonal regeneration throughout the optic nerve. Remarkably, DNMT3A inhibition also resulted in restoration of spatial visual function in adult mice following optic nerve crush injury. Integrated genome-wide DNA methylation and transcriptomic analyses, including singlenucleus RNA sequencing of RGCs, revealed selective DNA demethylation and reactivation of gene networks governing neuronal survival, axonal growth, and regenerative competence. Together, these findings establish DNMT3A as a central epigenetic switch controlling axon regeneration in the adult visual system. Strategic targeting of DNMT3A through gene therapy—based approaches represents a promising therapeutic paradigm for optic neuropathies and broader neurodegenerative disorders, highlighting epigenomic reprogramming as a viable strategy to promote neural repair and functional recovery.

Biography:

Wai Lydia Tai, PhD is a Postdoctoral Fellow at the Schepens Eye Research Institute, Massachusetts Eye and Ear/Harvard Medical School. Her research centers on the mechanisms underlying neurocircuitry formation and repair, with an interest in their translational potential. She earned her B.A. in Cell and Developmental Biology from the University of California, Berkeley, and her M.Phil. and Ph.D. in Neuroscience from the University of Hong Kong, where she studied neuropathic signaling in the CNS. Her current work focuses on epigenetic regulation of neuroregeneration following optic nerve injury, with an emphasis on retinal ganglion cell repair Low-Cost AAV Viral Vectors Can be Manufactured Profitably: Engineered HEK-293 Cells and New Tools Combine to Yield Stable, High-Productivity Producer Cells.

Genetic testing in clinical trials: The need for robust framework focused on the patient

Rachel Smith, Vice President, Rare and Genetic Diseases, Parexel International

Abstract:

There are over 10,000 distinct types of rare and genetic disease impacting >400 million patients globally, with over 80% linked to known or suspected genetic causes1, presenting significant opportunity for development of targeted genetic therapies. To date, gene therapy has been restricted to monogenic disorders as these typically present more “straightforward” therapeutic either through gene replacement, modification or gene repair2. For example, the first successful gene therapy to treat a patient with the monogenic condition, Severe Combined Immunodeficiency due to Adenosine Deaminase Deficiency (ADA-SCID), utilised ex vivo modification of the patient’s own CD34+ cells via gamma retroviral vector on 15 September 19903. The first ex vivo CD34+ gamma retroviral gene therapy, Strimvelis, was only approved in May 20164 for the same condition with only a handful of ex vivo cell-based gene therapies approved since 2016 as the research world tackled the complex challenges presented by gene therapies including immunogenicity, delivery to target tissues, off-target effects, insertional mutagenesis and high cost of development2.

However, with the evolution of increasingly complex gene editing techniques along with nucleic acid therapeutic strategies and the promise of AI, we are approaching the possibility of polygenic disease treatments alongside the reality of personalised individual therapies e.g. Baby KJ5. With this technological expansion, there is a greater need to obtain in depth genetic data at an individual patient level and at population levels to inform the viability of these therapies and future research direction in rare disease.

Obtaining genetic data at an individual level for drug development companies requires a robust infrastructure built around compassion with the patient at the center. This is sensitive data and may directly impact the future of their care. Whether that is inclusion or rejection from a clinical trial, a non-viable variant for gene therapy, updated diagnosis due to more advanced testing methods, poorer prognosis, or the impact of inheritance. Here we discuss the critical framework needed for clinical trials requiring genetic testing focused on patient engagement.

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From Consultation to Co-Creation: Building Durable Patient Partnerships in Gene Therapy Development

Presenter and Co-author names Melissa Penn (and other panelists)*

Affiliation (Institution/organization name, Country) Bayer Pharmaceuticals, USA

Abstract:

Gene therapies offer transformative potential across both rare and complex non-rare conditions, and participation often requires patients and families to make difficult, high-stakes decisions with long-term implications. Across medicine and public health, lived experience expertise is increasingly recognized as essential and equal in importance to clinical and scientific expertise, signaling a meaningful culture shift in how decisions are informed. In gene therapy—where interventions are transformative, risks are significant, and development timelines are long—this shift must be intentionally reflected: patients are not only the *why* of clinical research, but the *how* and the *what*. Grounded in the principle of **"Nothing About Us Without Us,"** gene therapy development requires trust-based partnerships with patient communities that move beyond episodic engagement toward sustained co-creation.

This panel will consider how durable collaborations and partnerships with patient communities can be intentionally designed, operationalized, and sustained through co-creation across the gene therapy development lifecycle. As part of the discussion, examples may be used to illustrate how standing patient councils, grassroots ambassador models, and advocacy partnerships can inform strategy and support high-stakes decision-making.

Topics for discussion may include:
• Designing patient partnerships aligned with scientific, operational, regulatory, and strategic objectives
• Integrating co-creation into protocol design, recruitment strategies, and trial execution
• Building trust and continuity across multi-year development programs
• Translating patient insights into measurable impacts on enrollment, retention, and representativeness

Biography:

Melissa Penn leads Patient Engagement R&D at Bayer Pharmaceuticals, shaping strategic patient partnership models across complex therapeutic areas, including cell and gene therapy. With more than 20 years of experience spanning rare and non-rare conditions, she focuses on co-creation with patient communities across research design, trial execution, and cross-sector collaboration. Melissa has helped build standing patient councils, advocacy partnerships, and scalable engagement frameworks that inform strategy, strengthen representativeness, and support long-term development. She is an attorney and holds a Master of Public Health.

Building durable patient partnerships

Daya Verma, Novartis, USA

FDA Inspection & GMP Expectations for Human Gene Therapy Products

Alonza Cruse

Abstract:

Gene therapy products face heightened FDA scrutiny due to complex manufacturing, safety risks, and variability. This session highlights key inspection trends, common GMP gaps, and challenges in comparability, potency assays, and validation. Practical strategies for inspection readiness and building a mature, compliant quality system will support reliable, safe gene therapy production.

Title Pre-Approval (PAIs) vs Surveillance Inspections and Inspection Readiness Best Practices

Deyaa Shaheen, Founder and CEO of XFDA Pharma Advisors Consulting Services

XFDA Pharma Advisors Consulting Services

Abstract

Effective preparation for U.S. Food and Drug Administration (FDA) inspections is essential for maintaining a compliant pharmaceutical manufacturing environment. This presentation highlights the key differences between **Pre-Approval Inspections (PAIs)** and **Surveillance Inspections**, emphasizing how each serves a distinct regulatory purpose. PAIs focuses on verifying readiness for commercial product approval, including data integrity, and readiness for manufacturing. In contrast, surveillance inspections evaluate the ongoing state of cGMP compliance across the facility, concentrating on the robustness of the quality system, documentation practices, and long-term process control.

The session will outline tailored **inspection-readiness strategies effective interaction with FDA investigators**, including best practices for communication, managing document requests, responding to questions accurately, using daily briefings to maintain alignment, and avoiding common behaviors that may unintentionally raise concern. Attendees will gain a structured understanding of how to prepare proactively, establish confident and consistent investigator interactions, and maintain a state of inspection readiness that supports both regulatory expectations and operational excellence.

Biography:

Accomplished senior compliance executive with nearly 25+ years of global pharmaceutical and regulatory experience, including roles as FDA Drug Investigator and CDER Compliance Officer. Expert in CGMP compliance, inspection readiness, data integrity, and global health authority interactions. Supported 50+ FDA inspections and led audit programs across 100+ global sites. Skilled in FDA 483/Warning Letter strategy, regulatory meeting preparation, QMS remediation, and CMC/data integrity reviews for NDA, ANDA, and BLA submissions. Known for strengthening quality systems, reducing compliance risks, and fostering a culture of quality and patient safety.

A Reg-CMC Roadmap for Driving Concurrent US and EU Approvals for Gene Therapies

Rajiv Gangurde, Ph.D., Vice President, TechOps, Cell & Gene Therapy, Parexel

Abstract:

Given the increasing focus on the development of gene therapies (GT) for global markets, a harmonized regulatory approach to CMC requirements is highly desirable. Harmonization could potentially accelerate access and reduce development costs by minimizing or eliminating duplicative work. For developers seeking GT approvals in both the US and the EU, it is essential to understand the similarities and differences between the two regulatory systems, and to develop strategies that align with the expectations of both agencies. This is particularly important for CMC activities, as early decisions related to process, analytical, and manufacturing strategies can impact licensure as well as commercialization. This presentation focuses on areas of similarities and key differences in the regulatory framework and requirements of FDA and EMA, with an emphasis on aligning CMC and regulatory strategies at various stages of development.

Proof of Concept to IND Readiness: How IND-Enabling Materials De-risk Regulatory and Funding Milestone

Emily Moran, 3LB Consulting, LLC, USA

A Collaborative Path to Next-Gen Viral Vector and Plasmid Production: Intensified, Monitored, and Affordable

Rachel Legmann

Abstract:

The growing demand for viral vectors and plasmids requires manufacturing that is robust, scalable, and economically sustainable. This collaborative work demonstrates how optimized technologies, partnerships, and advanced analytics can reshape next-generation nucleic acid and vector production. By integrating intensified upstream processing, real-time monitoring, automation, and streamlined downstream operations, we present a path to higher productivity while maintaining quality and compliance.

A case study using KrosFlo® TFDF®-based perfusion shows a 4-fold increase in cell mass and >4-fold higher cell-specific LVV productivity, enabled by efficient cell retention and low shear. Continuous cold harvest preserved LVV infectivity, while integrated continuous affinity captures simplified purification and improved yield.

Automated Process Control further reduced operational error and dramatically improved plasmid DNA purification, cutting processing time from 17 hours to 5.7 hours (a 298% efficiency improvement), highlighting its impact on reproducibility and throughput.

Overall, the successful implementation of the RS20 system and KRM chromatography demonstrates how automation, intensification, and monitoring enable scalable, high-quality, and affordable plasmid and vector manufacturing.

Issues and Crisis Management: Staving Off Disastrous Consequences: Preparedness and Strategic Approaches

Tristan Jervis, Impact Shine Communications, UK

Overcoming Barriers to Cell and Gene Therapy Commercialization

Curt Kugel1, Meghan McDonald1*

Biography:

Curt received his undergraduate degree in Cell Biology and Molecular Genetics from the University of Maryland, and a PHD in Genetics & Cancer Biology from Thomas Jefferson University. He performed his post-doctorate research on age-related responses to immune-checkpoint inhibitors at the Wistar

Institute before joining ZS Associates. He is now one of the leaders of the ZS Cell, Gene and Advanced Therapy vertical and has partnered with biotech and large pharma companies to successfully commercialize cell, gene and other advanced therapies across oncology, rare disease and chronic conditions for a variety of advanced therapy modalities both in market and in development.

Collaboration and Partnerships — A Parent and Patient Advocacy Perspective

Amanda Moore

Abstract:

The future of gene therapy depends not only on scientific innovation but also on trust, communication, and authentic collaboration with the patient communities these therapies are designed to serve. From the parent and advocacy perspective, partnerships between researchers, industry, and families are not peripheral — they are foundational to achieving meaningful, equitable outcomes.

This presentation will explore how patient advocacy organizations like the Angelman Syndrome Foundation (ASF) are redefining collaboration by integrating patient voices at every stage of therapy development — from study design and clinical readiness to real-world data collection and post-approval support.

Drawing from ASF's experience in building global clinical networks, family engagement models, and pre-competitive research partnerships, this talk will highlight:

- How co-designing studies with caregivers improves enrollment, retention, and relevance.
- The role of data-sharing and infrastructure (such as the LADDER database) in accelerating therapeutic readiness.
- Lessons learned from industry collaborations that prioritize transparency and mutual accountability.
- The human side of partnership — bridging the gap between hope, science, and lived experience.

Ultimately, the presentation emphasizes that progress in gene therapy is not measured solely in clinical milestones, but in the strength of the ecosystem we build together — one that listens, learns, and leads with compassion and shared purpose.

Analytical QC and potency assurance challenges

Patrick Starremans, Solid Biosciences Inc , USA

Analytical QC and potency assurance challenges

Hongwei Zhang , HZ Independent BioConsulting , USA

José Vázquez, Key Account Manager , c-LEcta GmbH ,Germany

Poster title: High Salt, High Impact: An Endonuclease Engineered for High-Efficiency DNA Removal in Viral Vector Manufacturing Abstract: In the manufacturing of viral vectors for cell and gene therapy and vaccine applications, ensuring the safety and efficacy of the final therapeutic product is of utmost importance. DNA contaminants resulting from lysis of genetically transformed cells used for virus production pose the potential threat of being immuno- or oncogenic.1,2 Therefore, the FDA has set acceptable limits for the concentration (>10 ng/dose) and size (>200 base pairs) of residual host cell and plasmid DNA in the final drug product.3,4 Endonucleases, which can break down all forms of nucleic acids into smaller fragments without affecting viral particle integrity, are used to meet this regulatory requirements. Currently, endonucleases from Serratia marcescens are the industry standard for enzymatic nucleic acid removal. The use of these enzymes not only enhances the safety profile of the viral vector-based therapeutics but also improves overall process efficiency by preventing the formation of aggregates and viscosity caused by presence of extracellular DNA. This work presents the performance a novel salt-tolerant enzyme, an engineered variant of the wild-type Serratia marcescens endonuclease specifically designed for efficient DNA removal at elevated salt concentrations and process-relevant pH conditions offering flexibility in viral vector purification processes. 1 Sheng-Fowler L, Lewis AM, Peden K. Issues associated with residual cell-substrate DNA in viral vaccines. Biologicals. 2009;37(3):190-195. doi:10.1016/j.biologicals.2009.02.015 2 Singh SK. Impact of Product-Related Factors on Immunogenicity of Biotherapeutics. J Pharm Sci. 2011;100(2):354-387. doi:10.1002/jps.22276 3 US Food and Drug Administration; Rockville, MD: 2010. Guidance for industry: Characterization and Qualification of Cell Substrates and Other Biological Materials Used in the Production of Viral Vaccines for Infectious Disease Indications. 4 US Food and Drug Administration; Rockville, MD: 2020. Guidance for industry: Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications.

Building Scalable and Execution-Ready GT Manufacturing and Supply Chains

Mihir Shukla, Principal Consultant

Affiliation: Biopharma Consulting Solutions, LLC, USA

Abstract:

Gene therapy manufacturing and supply chain models are rapidly maturing as programs move from early clinical development into late-stage development and commercialization. Earlyphase approaches that rely on bespoke processes, limited supplier redundancy, and informal lifecycle planning are increasingly unsustainable as demand, regulatory expectations, and operational complexity rise.This session provides a practical view of the current gene therapy manufacturing landscape and the supply chain constraints shaping timelines and cost. Key pressures include viral vector capacity and scheduling volatility, long lead times for critical raw materials and single-use components, limited supplier qualification depth, and concentrated risk from single-source dependencies. Sponsors must progress quickly while maintaining phase-appropriate quality systems and CMC discipline.Drawing on real-world program experience across AAV and other viral vector platforms, the presentation will highlight five practical levers that are becoming central to successful execution: early integration of CMC and supply chain planning; risk-based CDMO selection and governance; modular and flexible manufacturing models; fit-for-phase automation to improve consistency, throughput, and data integrity; and alignment with evolving FDA thinking on flexible, risk-based CMC expectations for cell and gene therapies.Attendees will gain a clear set of decision points and a practical framework to reduce late-stage surprises while preserving speed and innovation.

Biography:

Mihir N. Shukla is Founder and Principal Consultant at Biopharma Consulting Solutions LLC, advising biotech and pharmaceutical companies on CMC strategy, quality systems, and manufacturing execution across clinical and commercial stages. He has 15+ years of experience supporting complex gene therapy and biologics programs, including CDMO oversight, tech transfer, inspection readiness, and risk-based planning. Mihir works closely with executive teams to build scalable, stage-appropriate manufacturing strategies that preserve speed while reducing late-stage risk.

Low-Cost AAV Viral Vectors Can be Manufactured Profitably: Engineered HEK-293 Cells and New Tools Combine to Yield Stable, High-Productivity Producer Cells

Larry Forman

Abstract:

Current viral vector manufacturing methods are expensive, limiting gene therapy access and discouraging pharmaceutical investment for developing new treatments. Furthermore, large numbers of doses required for treating common diseases cannot be manufactured at any cost using current methods. Our breakthrough technologies—engineered high-productivity cells and non-leaky, inducible promotors—enable efficient, large-scale production of AAV, overcoming previous barriers of

cost and limited supply. We are also implementing the use of landing pads to shorten the time required to create inducible, stable producer cells. Our several AAV production technologies will be presented.

Building a Scalable, AI-Enabled AAV Manufacturing Platform: From Molecular Design to Commercial-Ready Production

Brian Tomkowicz

Abstract:

As AAV gene therapies expand toward systemic dosing and larger patient populations, manufacturing platforms must evolve beyond incremental process optimization. Sustainable commercialization requires integrated control across plasmid engineering, cell-line performance, scalability, analytics, and safety monitoring—supported by data-driven decision frameworks. We present a fully integrated, serotype-agnostic AAV manufacturing platform engineered for operational scalability and regulatory alignment.

Upstream robustness begins with rational plasmid architecture designed to enhance ITR stability and reduce recombination risk during amplification. Production is enabled by a monoclonal, serum-free suspension HEK293 cell line (4G9) optimized for high-density transient transfection and scalable bioreactor performance. The platform demonstrates consistent productivity and comparable critical quality attributes across multiple serotypes, with scalability from 2 L benchtop through 50 L pilot scale and readiness for larger commercial volumes.

Downstream operations incorporate AEX-based capsid enrichment strategies to improve empty:full ratios while maintaining process yield. To accelerate release readiness and in-process control, we implemented a GMP-qualified mass photometry assay enabling rapid, label-free quantification of capsid populations. Safety oversight is further strengthened through a GMP-compatible rcAAV detection assay with high sensitivity across serotypes.

To enhance operational efficiency, we are integrating AI-driven analytics in collaboration with invited industry partners to support process optimization, data harmonization, and predictive performance modeling across the manufacturing lifecycle.

This lifecycle-integrated, data-enabled platform positions AAV are not manufacturing as a collection of unit operations, but as a coordinated system designed for scale, speed, compliance, and commercial sustainability—supporting the next generation of high-dose and systemically delivered gene therapies.

Selective Removal of Early-Eluting Genome-Containing AAV Species with Elevated Residual DNA via Optimized Anion Exchange Chromatography

Nermin Ibreljic, Sarepta Therapeutics, USA

A Modified CRISPR Approach Using XIST for the Treatment of Down Syndrome

Volney Sheen, Harvard Medical School, USA

Frontiers of Gene Therapy: Trends and Opportunities from Payload through Scale Up

Anis H. Khimani, Ph.D., Senior Manager, Global Product Management, Antylia Scientific

Abstract:

Cell and gene therapy modality, including RNA therapies, continues to advance as evident from the pipeline. With 4,341 therapies in the pipeline and more than 3,200 trials underway globally, advancements in gene therapy are dynamic[1]. Over the past 20 years, 38 gene therapies have been approved globally. Oncology and rare disease continue to dominate the pipeline. Collective and collaborative efforts across various functional groups along the value chain within an organization as well as external partnerships are critical to drive progress. In this talk, opportunities and challenges in context of the efforts to drive success will be discussed.

Manufacturing and Supply Chain Strategies

Michael DiBiasio-White, ViroSpark BioConsulting , USA

Manufacturing and Supply Chain Strategies

David Greenwald , Deerfield Management,USA

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