Reporting from the Cutting Edge of Innovation in Cell and Gene Therapies

Priyanka Gupta, Ann Ablavskaya, Paul Cashen, Laura Falcone, Maya Fuerstenau-Sharp, Shanya Jiang, Alengo Nyamay'Antu, Bettina Renker, and Mukund Srinivasan

Hosted at the MassBio Hub in Cambridge, MA, the 2025 Sartorius Cell and Gene Therapy (CGT) Forum brought together key experts from across the industry for a day of presentations, panel discussions, and networking focused on collaboration, innovation, and evolving market dynamics for advanced therapies. Participants identified cost and funding pressures, scalability constraints, and regulatory complexity as the field's most pressing challenges — themes that shaped the discussions throughout the day.

"This forum is the place where transformative science happens," said Maurice Phelan, president of Sartorius North America. He went on to say that collaborations formed in the room would drive the next breakthroughs in CGTs.

The forum began with a plenary session to bring all attendees together for interactive panel discussions and fireside chats on shared industry challenges and opportunities. In the afternoon, attendees were divided into two parallel tracks — on cell and gene therapies, respectively — to dive further into modality-specific advances.

The Economics of CGT Innovation

Speakers across the opening sessions described a field that is shifting out of early exploration and into commercial, asset-driven growth. **Carl Schoellhammer (DeciBio)** characterized the CGT industry as entering its "teenage years," with the field moving from a proliferation of assets toward durable commercial franchises. Despite that unanimous observation, the path to broad adoption remains unclear. Awareness is still a major obstacle to overcome, particularly in clinical settings. "If a physician doesn't know what it is, they're not prescribing it," Schoellhammer said, pointing out that awareness and access now shape adoption more than therapeutic potential alone.

The economics of CGT maturation adds further nuance. **Philip Vanek (Redline Bioadvisors and International Society of Cell and Gene Therapy, ISCT)** emphasized the capital intensity of CGT programs, saying that "capital is the fuel for the rocket." Although launching a therapy can cost hundreds of millions of dollars, early stage financing is paradoxically constrained. As Vanek explained, it can be easier to raise $50–100 million than it is to raise $5–10 million. That gap risks suppressing high-potential innovations before they can reach clinical inflection points. He also underscored how regulators, value frameworks, and manufacturing choices all influence global patient access, pointing out that companies need to "begin with the end in mind" because many clinical studies for CGTs already encompass most of the intended treatment population.

Anthony Ting (Kiji Therapeutics) described how scientific ambition must be paired with commercial discipline. He encouraged early stage companies to focus on the whole picture, including team

composition, patient-advocacy engagement, and a clear target product profile (TPP) that integrates process scalability with key considerations from payers. Ting's case study on engineered mesenchymal stem cell (MSC) programs highlighted a persistent tension: Academic breakthroughs often underestimate the importance of quality. Variability in MSC source, culture conditions, and cryopreservation can influence therapeutic outcomes fundamentally, as evidenced by findings from his previous company, Athersys. In trials for stroke and acute respiratory distress syndrome (ARDS) indications, patients receiving higher viability cell products have demonstrated more favorable responses. Such findings have driven the company to prioritize technologies that support consistent, on-demand cell preparation.

All speakers agreed that CGTs are progressing toward commercial maturity, as indicated by continued consolidation and increasing therapeutic awareness and adoption. However, sustaining today's rate of innovation depends on aligning clinical adoption, financing, and development strategy. As Vanek noted, cost and value must be considered together. "Before CAR-T [chimeric antigen receptor T-cell therapy], one patient cost the system $10 million," he said, underscoring that the value created by transformative therapies is substantial. But future affordability hinges on improved efficiency rather than downward price pressure alone.

EXPERT INSIGHT:

"If a physician doesn't know what it is, they're not prescribing it."
—Carl Schoellhammer, DeciBio

Enabling Compliance, Reliability, and Scalability

Regulatory and manufacturing discipline surfaced as another major theme. **Doli Patel (Sartorius)** noted that although speed matters, success relies on a coherent regulatory strategy that is built around product potency and end-to-end process control. Patel stressed that chemistry, manufacturing, and controls (CMC) requirements remain one of the most difficult constraints, requiring future-proof designs, seamless scale-up, robust analytics, and digitalization capable of withstanding inspection (Figure 1). Her advice was clear: Understand compliance holistically, and do not rely on ad-hoc regulatory negotiations. Only rigorous, data-driven proposals can advance development meaningfully.

Figure 1: Asset value propositions and the cost of compliance (courtesy of Doli Patel, Sartorius)

Basak Clements (biomatria) reinforced that message in her presentation on materials science, pointing out that raw materials often are undervalued during early development despite being defining factors for CMC success. "Think more critically about materials," she said, noting how insufficient control of critical material attributes can lead to deviations, comparability failures, and costly batch losses. Early stage attempts to save on

materials often result in far more expensive regulatory and operational burdens later. Clements highlighted five priorities shaping the field: rising regulatory expectations, advanced analytics, harmonized standards, supplier collaboration, and material innovation. “Assessing and controlling your material risks early on will save you a lot of pain down the road,” she said.

EXPERT INSIGHT:

“Capital is the fuel for the rocket.”
—Philip Vanek, Redline Bioadvisors and International Society of Cell and Gene Therapy, ISCT

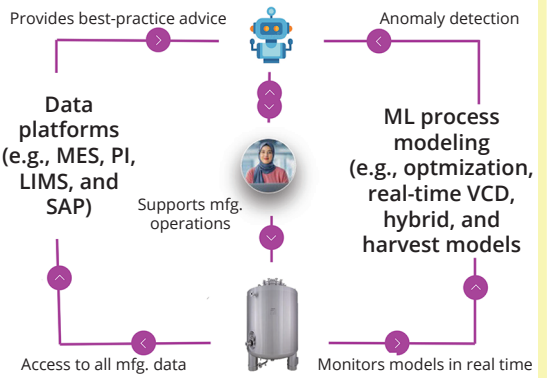
Sanjay Srivastava (Accenture) stated that inefficiencies and underused capacity also are driving much of the cost burden in manufacturing (Figure 2). Schoellhammer added that in vivo strategies might eventually reduce dose requirements to the “single-digit thousands,” potentially easing some scalability challenges.

Together, the speakers agreed that durable, compliant, and scalable CGT manufacturing relies on an integrated approach to quality by design (QbD), raw-material control, quality systems, and operational efficiency. Scientific innovation can catalyze development, but executional rigor keeps programs viable.

Digital Transformation and Broadening Patient Access

The session emphasized the transformative potential of AI and digital infrastructure, particularly for improving access and long-term sustainability. Srivastava outlined how agentic artificial intelligence introduces agents that monitor processes, detect deviations, and recommend corrective actions, creating a “self-healing” manufacturing environment. In one example, generative AI evaluated hundreds of thousands of CAR-T batch records to identify lineage purity as the main driver of out-of-specification (OoS) results — work that previously required months of manual analysis. “AI did the correlation and causality for us,” he said, highlighting how advanced computational approaches accelerate insight discovery.

Figure 2: Workflow of Accenture viral-vector–manufacturing artificial intelligence (AI) digital assistant (courtesy of Sanjay Srivastava, Accenture);



LIMS = laboratory information management system, MES = manufacturizng execution system, mfg. = manufacturing, ML = machine learning, PI = process intensification, VCD = viable cell density (**5**)

EXPERT INSIGHT:

“Assessing and controlling your material risks early on will save you a lot of pain down the road.”
—Basak Clements, biomatria

Setting the Standard in Cell and Gene Therapy, Together

Simplifying Progress

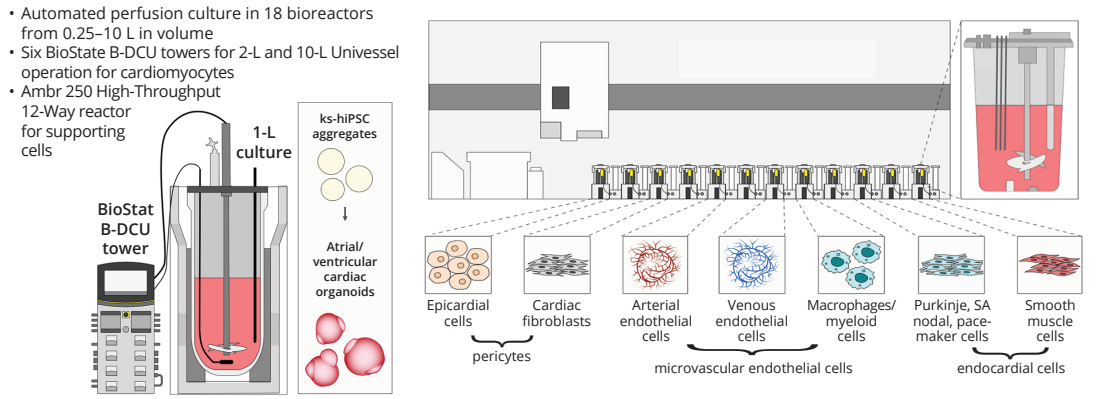
Quality-driven Media and Reagents for Your Next Breakthrough

From cell culture media and transfection reagents to recombinant proteins and biopreservation solutions, rely on our extensive portfolio to support safe, efficient and cost-effective advanced therapy production.

To request a sample, visit
https://sar.to/requestyoursample

SARTORIUS

Figure 3: Production of 13 isogenic cell types of the human heart at whole organ scale (courtesy of Mark Skylar-Scott, Stanford University); hiPSC = human induced pluripotent stem cell, SA = sinoatrial



Vanek extended the discussion to regulatory and operational opportunities, noting how AI could streamline comparability assessments and filings while supporting consistent manufacturing. Patel reinforced the expectation that digitalization will become increasingly important for regulatory acceptance, especially as machine learning (ML) guidance matures. But Srivastava emphasized that adoption in good manufacturing practice (GMP) settings will require robust safeguards such as data integrity, cybersecurity, bias mitigation, and documented intent from the start.

Together, those perspectives suggest that AI and digital infrastructure have transitioned from optional upgrades to critical strategies for expanding patient access. By tightening operations, reducing failure rates, and enabling more flexible deployment across treatment centers, digital tools have the potential to address both logistical and economic barriers.

Integrating Biology, Engineering, and Automation Drives Scalability

Mark Skylar-Scott (Stanford University) emphasized that three-dimensional (3D) bioprinting of organ-scale tissues requires teams to "think of the end before you start from the beginning." Using embedded 3D printing inside soft support gels, his group produces dense, perfusable cardiac tissues from induced pluripotent stem cell (iPSC) – derived cardiomyocytes.

His team optimized suspension culture of human iPSC derived aggregates using an automated Ambr 250 Modular 250-mL stirred tank bioreactor system in combination with MODDE multivariate-analysis software, achieving >4 million cells/mL with >95% viability (Figure 3). The optimized process parameters were scaled successfully to automated 1-L and 10-L Univessel stirred tank bioreactor systems.

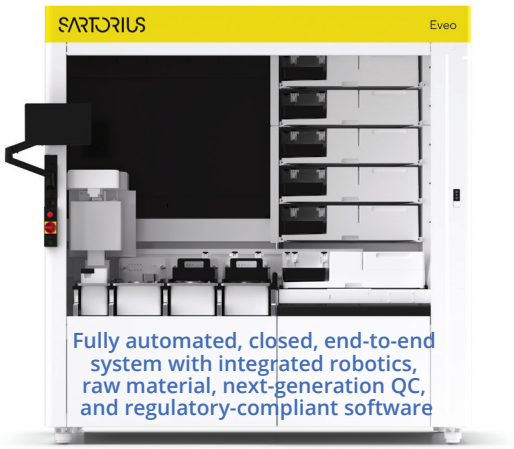
Chesney Michels (ElevateBio) presented his work leveraging the new Eveo platform from Sartorius to support low processing volumes, continuous perfusion, and real-time process monitoring (Figure 4). Describing a production process for T-cell receptor T (TCR-T) cells, he showed how the system simplifies user workflows, minimizes hands-on time, and reduces operator variability while maintaining consistent cell quality. Process optimizations that previously required multiple runs were completed "within a

EXPERT INSIGHT:

"Inefficiencies and underused capacity also are driving much of the cost burden in manufacturing."
—Sanjay Srivastava, Accenture

week." His work also highlighted how adopting the LipidBrick Cell Ready ready-to-use lipid-based gene delivery solution can achieve high T-cell receptor α constant (TRAC) knockout efficiency comparable to electroporation, strong cell viability, and robust cell expansion superior to electroporation.

Figure 4: Newly launched Eveo Cell Therapy Platform from Sartorius (courtesy of Chesney Michels, ElevateBio)



Operational Efficiency
Automation and modularity enable concurrent processing with optimized labor and footprints

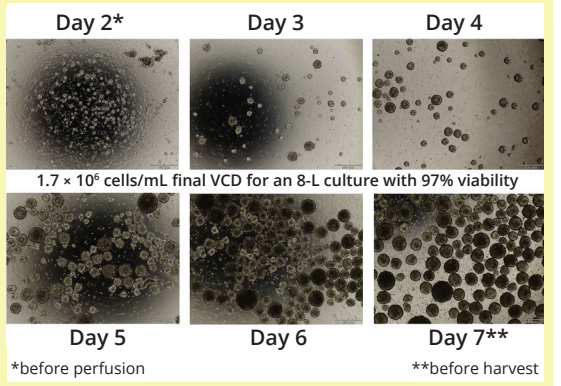
Digital Control
Real-time monitoring, batch-tracking, and quality oversight across parallel workflows

Risk Mitigation and Scalability
Lowers failure risk, supports regulatory approval, and scales to thousands of patient doses

Bioprocess Design and Analytics Address Variability and Scale

Another theme across the cell-therapy track was the persistent challenge of variability. **Sarah Gilpin (Sartorius)** emphasized that iPSCs require tailored, cell-line–specific manufacturing strategies because "it's not one-to-one." In collaboration with Cedars-Sinai Medical Center, Gilpin's team developed a GMP-ready working cell bank using an iPSC-specific medium to establish baselines for reproducible growth. By performing sequential studies in Ambr 250 bioreactors and 2-L suspension systems, the team refined aggregation and media-exchange parameters before scaling to 10 L, demonstrating a structured path toward quality-focused, scalable iPSC expansion (Figure 5).

Figure 5: Induced pluripotent stem cell (iPSC) expansion with continuous perfusion can be scaled to 10 L (8-L culture volume) with high viable cell density (VCD) and viability and without impact on pluripotent phenotype (courtesy of Sarah Gilpen, Sartorius).



EXPERT INSIGHT:

"Think of the end before you start from the beginning."
—Mark Skylar-Scott, Stanford University

Pierre Springuel (University College London) presented an evaluation of autologous CAR-T systems that reinforced variability as a defining barrier. "Regulatory approval relies on the ability to consistently manufacture ATMPs [advanced-therapy medicinal products]," he said, a standard that many legacy workflows struggle to meet due to manual and fragmented touchpoints and decentralized processes. As he described it, the Eveo platform

consolidates integrated perfusion, digital analytics, and automated sampling to increase reproducibility while shortening vein-to-vein timelines (Figure 6).

Sarah Nikiforow and Julie Porter (Dana-Farber Cancer Institute) discussed clinical variability. They presented real patient scenarios to illustrate how manufacturing quality, logistics, and patient status interact in decision-making across different stages of the value chain. Their team emphasized the importance of rapid testing, point-of-care manufacturing, and tight clinical-manufacturing feedback loops to manage the unpredictability that is inherent in autologous therapies. Those same structures serve as engines for translational research, enabling rapid iteration and increased control over therapeutic readiness.

New Delivery Models Accelerate Cell Therapies

The last major theme from the cell-therapy track focused on evolving delivery models and the infrastructure required to support next-generation cell therapies. Many projects presented at the forum demonstrated the benefits of codevelopment models that blend the strengths of academia and industry.

Springuel and Michels each showed how their respective groups worked with Sartorius to develop facilities that could accelerate iteration, specifically by providing access to automated, modular systems that reduce dependence on manual processes. Those models help bridge the translational divide by enabling teams to test manufacturing hypotheses in conditions that approximate regulated production environments.

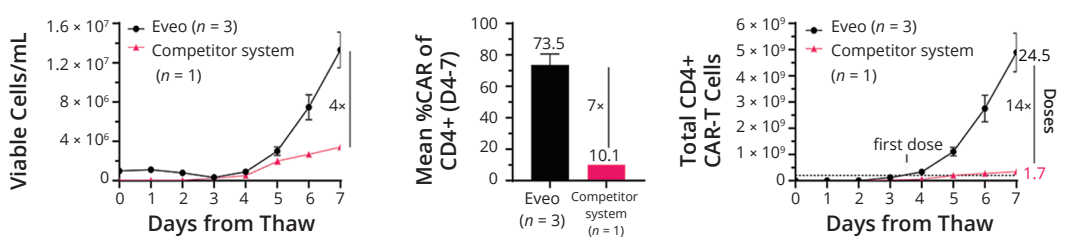
The most comprehensive example of a new delivery architecture came from Dana-Farber's cell-manipulation core facility, as presented by Nikiforow and Porter. Positioned as both a "cell pharmacy" and a translational engine, the facility enables point-of-care manufacturing, rapid product testing, and real-time alignment between clinical decisions and production constraints. The presenters emphasized that, as demand increases, so will the need for sustainable collaborations, infrastructure planning, and resource-sharing frameworks across academia and industry.

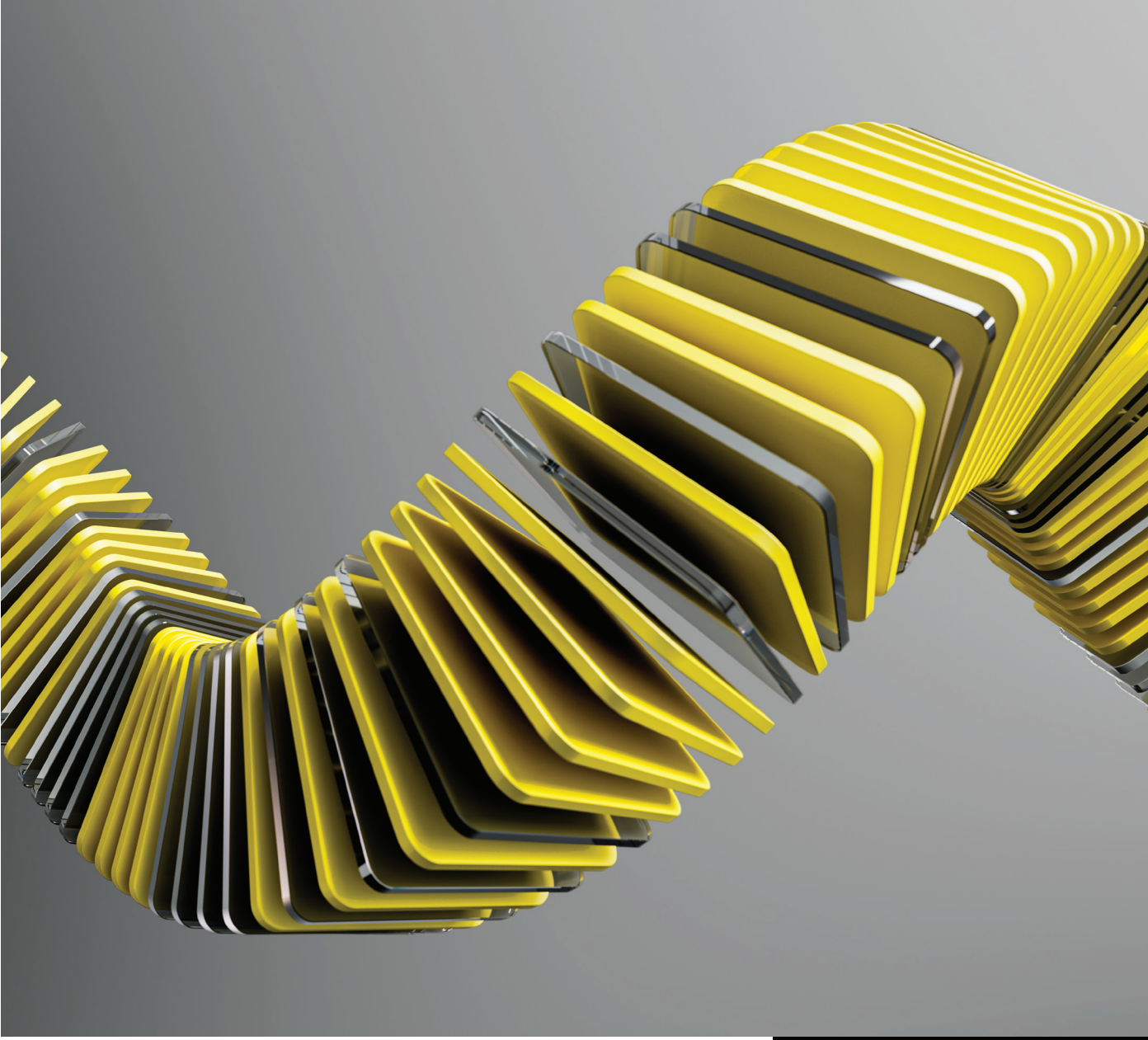
Redefining Gene Therapy for Scalability

The gene therapy track opened with a detailed examination of how tightly coordinated bioprocess engineering can transform adeno-associated virus (AAV) manufacturing. **Trent Lyman (Matica Biotechnology)** presented an end-to-end AAV8 platform built to perform consistently across scales. Using a suite of bioreactors — Ambr 250 HT, Univessel SU, and Biostat STR — combined with critical raw material FectoVIR-AAV from Sartorius, his team established comparable cell-expansion and viral titers from development scale through a 50-L demonstration run (Figure 7A and 7B). For downstream processing, using Sartoclear depth filters, Hydrosart tangential-flow filtration (TFF) cassettes, and CIMmultus SO3 and CIMmultus PrimaS chromatography monoliths resulted in strong impurity reduction and high full-capsid enrichment. Matica's work addressed core industry bottlenecks in recovery, purity, and flexibility, illustrating how an integrated process architecture can support scalable viral-vector production.

Innovations in nonviral delivery expanded on the theme of engineering-driven scalability. **Hai-Quan Mao (Johns Hopkins University)** described how precise control and AI-guided design are enabling development of highly efficient nanoparticle systems. His team used LipidBrick lipid nanoparticles (LNPs) from Sartorius and developed kinetically controlled mixing approaches to overcome diffusion limitations in PEIpro–DNA transfection complexes, producing uniform, size-controlled nanoparticles at liter scale. By adding a kinetic modulator, the

Figure 6: The performance of the Eveo platform compared with competitor system performance (courtesy of Pierre Springuel, University College London); CAR-T = chimeric antigen receptor T cell, CD4+ = cluster of differentiation 4





Setting the Standard in Cell and Gene Therapy, Together

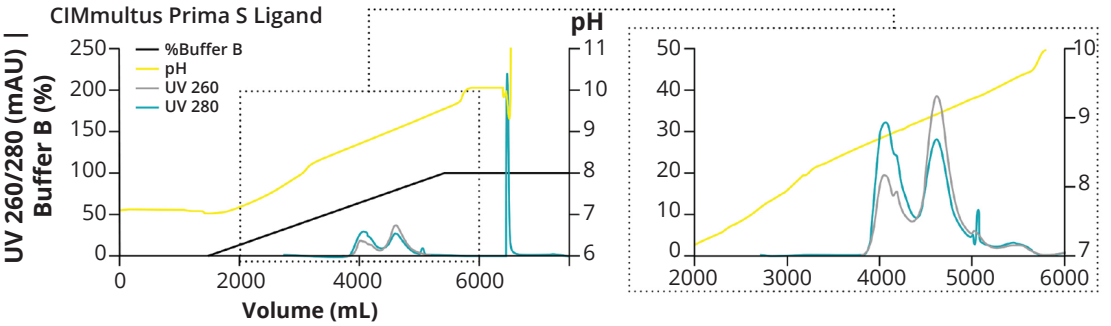
Enhance your process with solutions driven by reproducible performance, scalability, and the quality needed for regulatory approvals.

Set the Standard With Us
sartorius.com/cell-gene-therapy

Simplifying Progress

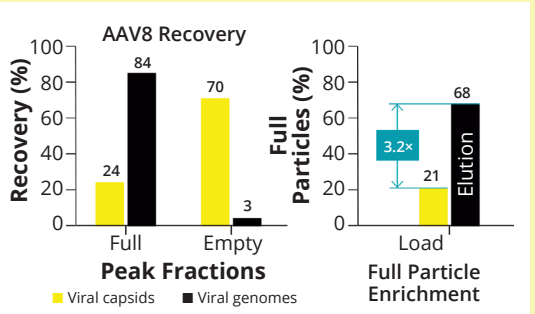
SARTORIUS

Figure 7A: Separation of empty and full particles using CIMmultus PrimaS multimodal chromatography ligand (courtesy of Trent Lyman, Matica Biotechnology); UV = ultraviolet



team controlled particle size, significantly improving performance — achieving a five-fold increase in DNA uptake and a 10-fold reduction in bioreactor volume. Mao also showcased how ML drove optimization of LNP helper compositions, identifying variants that enhanced stability and extended mRNA expression. In animal studies, the resulting LNPs supported durable immune activation and sustained transgene expression beyond injection sites. To improve analytics, his team developed fluorescence-based "cell CT scans" to map cargo distribution across populations of nanoparticles — an approach that could reshape quality control for emerging nonviral modalities.

Figure 7B: Separation of empty and full particles using CIMmultus PrimaS multimodal chromatography ligand (courtesy of Trent Lyman, Matica Biotechnology); AAV = adenoassociated virus



Standardizing and Modernizing Manufacturing Infrastructure

Miriam Monge (Sartorius) and **Dennis Powers (G-CON)** highlighted the importance of infrastructure innovation. They detailed a platform-based collaboration aimed at accelerating facility deployment and enhancing supply-chain resilience. Their joint approach integrates modular cleanroom construction with the Sartorius ProcessGo platform, creating standardized environments that developers can reuse across products and modalities (Figure 8). Monge noted that "85% of people say 'yes'" when asked whether they would adopt standardized solutions over customized ones, showing the industry's strong shift toward predictable, preengineered facilities.

Powers agreed, describing G-CON's modular platform as one that "facilitates standard, scalable, cost-efficient manufacturing." Beyond speed and repeatability, he emphasized sustainability as central to modern facility strategy. "Sustainability goes hand in hand with continuous improvement," he said, observing that both modular construction and integrated automation are linked to long-term operational efficiency. By reducing variability in facility design and shortening deployment timelines, modular systems offer a practical path toward manufacturing architectures that can adapt as pipelines grow and diversify.

Driving the Future of Gene Therapy Development

The gene therapy track concluded with a focus on data and digital intelligence. **Sandra Klausing (Sartorius)** highlighted that using AI, ML, and big data is "not only an opportunity for improvement, but a necessity to remain competitive." Despite rapid growth in data generation throughout bioprocessing, many organizations still struggle to convert raw data into meaningful process knowledge. Klausing shared several internal case studies illustrating how powerful analytics and structured data use

enable deeper understanding: optimizing human embryonic kidney cell–based lentiviral vector production through design of experiments, identifying metabolites that govern cell growth using high-resolution electrostatic ion-trap mass spectrometry and multivariate data analysis, and applying secretome or metabolome analytics to improve AAV productivity (Figure 9).

In those examples, Klausing emphasized that "the most difficult part is making sense of all these data" and that data-driven insights open new possibilities for process modeling, cell line development, and predictive molecule and media design. Her perspective echoed themes from previous speakers: Engineered platforms and modern facilities will reach their full potential only when paired with intelligent analytics that guide decision-making and reduce uncertainty.

Today's Breakthroughs Become Tomorrow's Therapies

The 2025 Sartorius CGT Forum illustrated a field that is converging rapidly on smart, scalable, and data-driven approaches to advanced therapies. Throughout the plenary session and modality-specific tracks, experts showcased innovations that are reshaping what is technically and operationally possible: organ-scale bioprinting, automated closed-system manufacturing, nonviral delivery, AAV platforms with robust full-capsid enrichment, AI-enhanced process understanding, and more. Despite the diversity of modalities and challenges, a consistent message became clear: Tomorrow's breakthroughs will rely on designing solutions with the end in mind, integrating strong analytics early, and building processes that can scale without sacrificing quality.

Looking ahead, multiple speakers said that progress increasingly will depend on collaboration, standardization, and intelligent automation. Academic centers, start-ups, and global manufacturers all underlined the need for adaptable platforms, digitally enabled workflows, and cross-disciplinary partnerships capable of reducing variability and accelerating translation. The technologies showcased throughout this forum represent more than incremental improvements: They form a foundation for making CGT therapies more accessible, more

Figure 8: Example of Sartorius ProcessGo platform using Biobrain Supervise software (courtesy of Miriam Monge and Dennis Powers); OPC = open platform communications, RM = remote monitoring, STR = stirred-tank reactor, UA = unified architecture

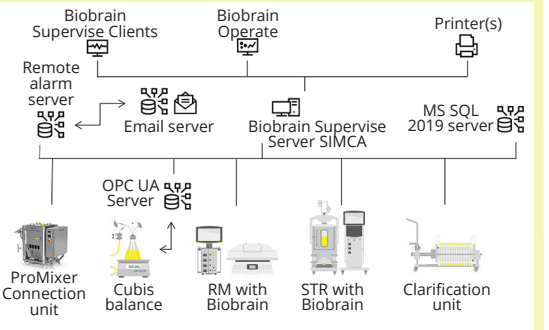
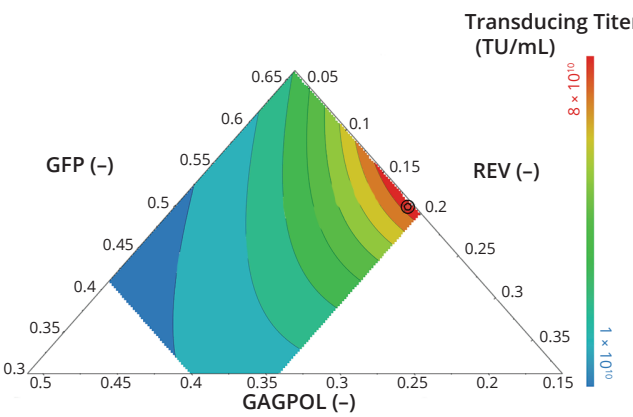
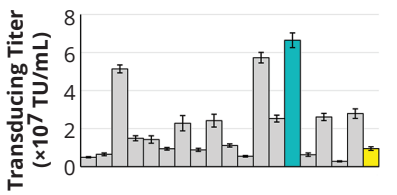
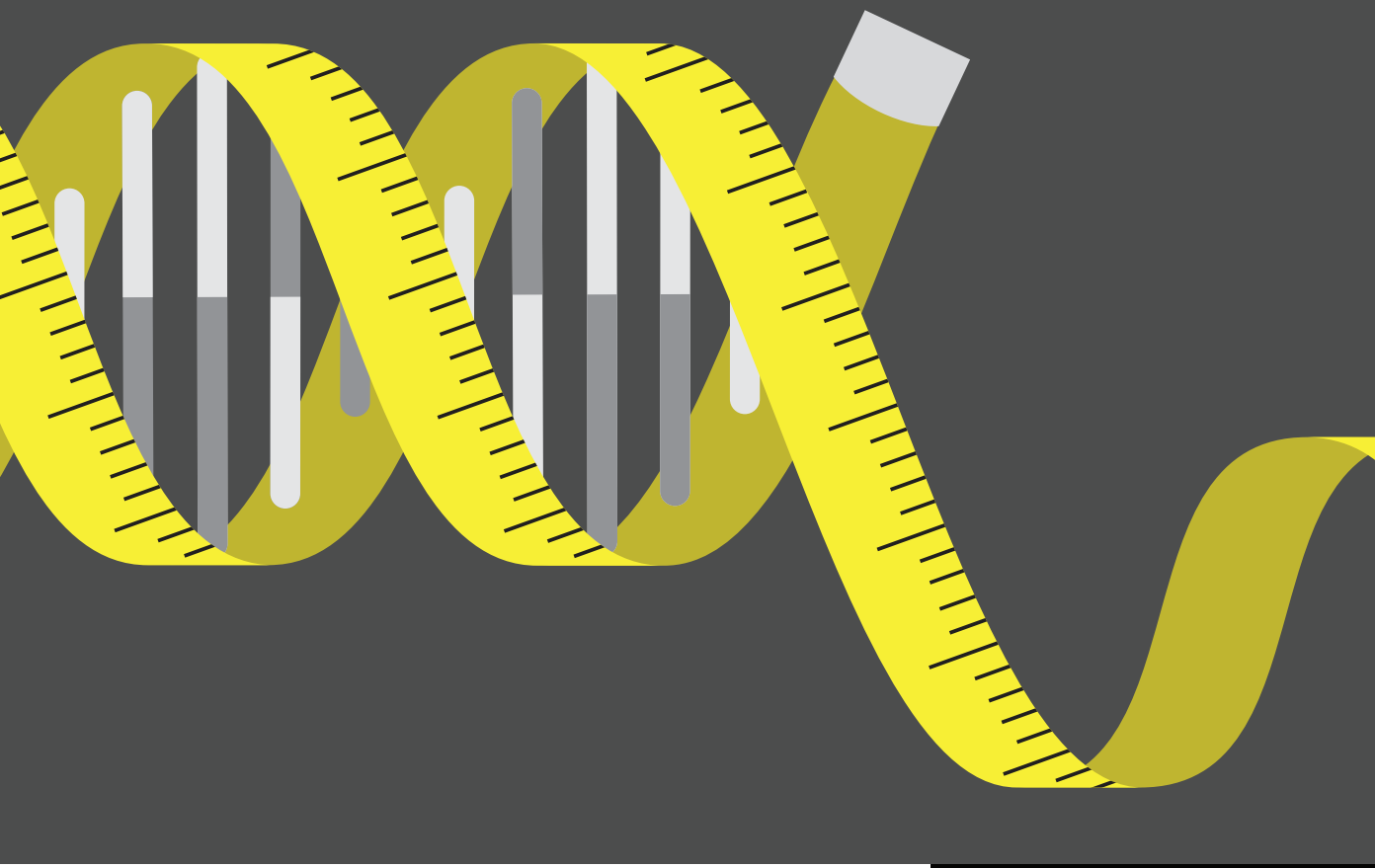


Figure 9: Ambr 15 optimization of plasmid ratios for three packaging plasmids and gene of interest (GoI) plasmid using a design-of-experiments (DoE) approach (courtesy of Sandra Klausing, Sartorius). Harvest was performed at 72 hours post-transfection, and transducing titer was analyzed by flow cytometry. Transducing titer was increased by a factor of seven (teal bar) compared with the default plasmid ratio (yellow bar). (TU = transducing unit)





Plasmid	Share (optimized)
GFP (Aldevron)	0.498
GAGPOL (Polyplus)	0.155
REV (Polyplus)	0.187
VSVG (Polyplus)	0.160



The Perfect Fit

Simplifying Progress

Comprehensive pDNA and mRNA Solutions Made For You

Nucleic acid processes vary widely in vector design, expression systems, purification constraints, and regulatory expectations. Sartorius provides comprehensive solutions designed to provide maximum flexibility throughout your pDNA and mRNA development journey — from discovery and pre-clinical stages to commercial manufacturing.

To learn more, vist
https://sar.to/theperfectfit

SARTORIUS

affordable, and more globally deployable than ever before. With continued investment in data, design, and community networks, the path from discovery to patient impact is set to shorten dramatically.

Acknowledgments

The following contributors are listed alphabetically: Basak Clements (biomatria), Laura Falcone (Sartorius), Maya Fuerstenau-Sharp (Sartorius), Sarah Gilpin (Sartorius), Sandra Klausing (Sartorius), Trent Lyman (Matica Biotechnology), Hai-Quan Mao (Johns Hopkins University), Chesney Michels (ElevateBio), Miriam Monge (Sartorius), Sarah Nikiforow (Dana-Farber Cancer Institute), Doli Patel (Sartorius), Julie Porter (Dana-Farber Cancer Institute), Dennis Powers (G-CON), Carl Schoellhammer (DeciBio), Mark Skylar-Scott (Stanford University), Pierre Springuel (University College London), Sanjay Srivastava (Accenture), Anthony Ting (Kiji Therapeutics), Philip Vanek (Redline Bioadvisors and ISCT).

Further Reading

Cell & Gene Therapy Expert Collection: Q&A With Seven Experts [eBook]. Sartorius Stedim Biotech GmbH: Goettingen, Germany, 2025; https://www.sartorius.com/en/whitepaper-setting-standard-in-cell-and-gene-therapy-1642912.

Greatorex E. Autologous Cell Therapy: Can Integrated Platforms Break the Bottlenecks? *BioProcess Int.* 23(10) 2025: 42–44; https://www.bioprocessintl.com/sponsored-content/autologous-cell-therapy-can-integrated-platforms-break-the-bottlenecks-.

Kunze Küllmer M, et al. Cell and Gene Therapy Investment: Evolution and Future Outlook on Investor Perspectives. *Cytotherapy* 26(7) 2024: 672–680; https://www.isct-cytotherapy.org/article/S1465-3249(24)00064-1/fulltext.

Ho DLL, et al. Large Scale Production of Wholly Cellular Bioinks via the Optimization of Human Induced Pluripotent Stem Cell Aggregate Culture in Automated Bioreactors. *Adv. Healthc. Mat.* 11(24) 2022: 2201138; https://doi.org/10.1002/adhm.202201138.

ElevateBio. *Streamlining CRISPR-Cas9 Gene Editing With LipidBrick® Cell Ready* [poster]. ASGCT Annual Meeting: New Orleans, LA, 2025; https://elevate.bio/wp-content/uploads/2025/09/ElevateBio_Liposome_Poster.pdf.

Building an AAV8 Platform, Together: Lessons From a Collaborative Development Journey [white paper]. Sartorius AG: Goettingen, Germany, 2025; https://www.sartorius.com/en/white-paper-building-an-aav8-platform-1748470.

Hu Y, et al. Liter Scale Manufacturing of Shelf Stable Plasmid DNA/PEI Transfection Particles for Viral Vector Production. *Mol. Ther. Meth. Clin. Dev.* 32(1) 2024: 101194; https://doi.org/10.1016/j.omtm.2024.101194.

*Corresponding author **Priyanka Gupta** is senior bioprocess solutions (BPS) expert (priyanka.gupta@sartorius.com); **Ann Ablavskaya** is expert marketing and scientific copywriter; **Paul Cashen** is process expert for commercialization and optimization; **Laura Falcone** is senior manager of segment marketing for advanced therapy solutions (ATS); **Maya Fuerstenau-Sharp** is head of marketing, ATS; **Shanya Jiang** is segment marketing manager, ATS; **Alengo Nyamay'Antu** is segment marketing manager, ATS; **Bettina Renker** is BPS scientific events and advocacy expert; and **Mukund Srinivasan** is segment marketing manager, ATS; all at Sartorius.*