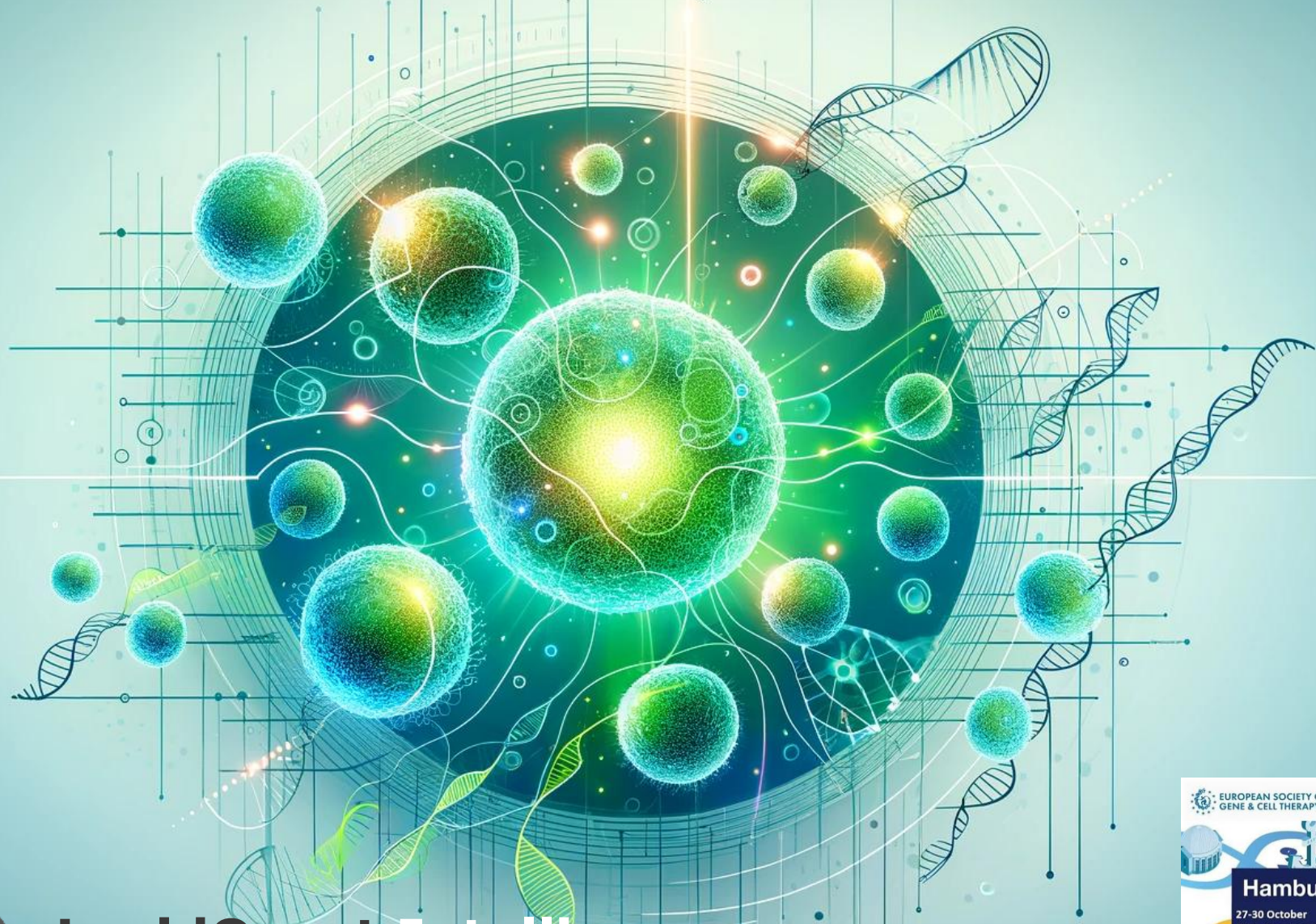


ESGCT 2026 Preview – Clinical and AI / ML



LucidQuest Intelligence
Accelerate your success



ESGCT 2026 Preview – Table of Contents

• <u>New Trends in Gene & Cell Therapy At ESGCT'26</u>	<u>3-5</u>
• <u>Key Sessions To Watch</u>	<u>6</u>
• <u>Industry-sponsored Sessions & Focus Areas At ESGCT 2026</u>	<u>7-14</u>
• <u>Competitive Landscape At a Glance at ESGCT 2026</u>	<u>8-9</u>
• <u>Key Industry-sponsored Sessions At ESGCT 2026</u>	<u>10-13</u>
• <u>New Entrants</u>	<u>14</u>
• <u>AI/ML Trends & Strategic Watchpoints At ESGCT 2026</u>	<u>15-18</u>
• <u>AI / Digital Health Angle At ESGCT 2026</u>	<u>16-18</u>
• <u>What We're Watching For Post-congress</u>	<u>19</u>
• <u>Get In Touch With LucidQuest</u>	<u>20</u>



ESGCT 2026 – New Trends in Gene & Cell Therapy (1/3)



- **Will AAV differentiation increasingly depend on delivery, safety and durability?**

- ESGCT 2026 will feature AAV research spanning capsid engineering, tissue targeting, host-vector interactions, immune responses and clinical translation. Presentations will cover AAV-based programmes in conditions including Duchenne muscular dystrophy (DMD), Dravet syndrome, Huntington's disease and Crigler-Najjar syndrome. The competitive focus will be on whether improvements in targeting and delivery can translate into sustained therapeutic effects while addressing safety and pre-existing immunity



- **In Vivo Gene Editing: Advancing Clinical Translation Beyond Ex Vivo Approaches**

- The programme will examine in vivo base editing, targeted genomic integration and other genome-editing approaches across metabolic, neurological and genetic diseases. Clinical proof-of-concept for in vivo base editing in PI*ZZ alpha-1 antitrypsin deficiency will be a notable focus, alongside work on Krabbe disease and multisystemic disorders. These presentations will help assess the progress of in vivo approaches and their potential advantages and challenges relative to ex vivo cell engineering





• **Non-Viral Delivery: Expanding Options for In Vivo Cell Engineering**

- Scheduled clinical data will span LGMD (ATA-200, ATA-100), Duchenne (GNT0004, RGX-202), OPMD (BB-301), Dravet syndrome (ETX101) and tauopathies (AVB-406), alongside first-in-human work in CTNNB1 syndrome. Sessions on cardiac and muscular gene therapy and two CNS & sensory sessions will carry much of this. The competitive question will be whether dose, durability and hepatic safety separate programmes targeting the same indications



• **CAR-T Innovation: New Approaches to Solid Tumours and In Vivo Cell Generation**

- The programme will examine CAR-T engineering, synthetic circuits, T-cell receptor design, immune-cell function and in vivo gene delivery. Solid-tumour sessions will explore approaches to tumour targeting, T-cell trafficking and the tumour microenvironment, while other presentations will investigate in vivo CAR-T generation through viral and non-viral delivery. The key area to watch will be how these strategies address the challenges of cell therapy in solid tumours





ESGCT 2026 – New Trends in Gene & Cell Therapy (3/3)



- **Manufacturing has become the largest single area of activity in the programme**

- The programme will cover AAV production, vector purification, HEK293 cell-line workflows, lentiviral manufacturing, serum-free T-cell processing and scalable non-viral delivery. Industry sessions from Sartorius, Lonza, PlasmidFactory and Cytiva will complement scientific presentations on manufacturing and development. Together, these discussions will highlight the importance of process consistency, production scale and development decisions in advancing gene and cell therapies toward clinical application



- **AI-Driven Design: From Vector Engineering to Regulatory Considerations**

- AI and machine learning will feature in AAV capsid engineering, sequence design, manufacturing optimisation and genome-editing safety prediction. The programme will also include a dedicated session on AI in advanced therapy medicinal product (ATMP) regulation. These topics will provide a view of how AI applications are expanding across therapeutic design and process development, alongside the need for appropriate validation and regulatory consideration



Key Sessions To Watch

Date	Session Title (short)	Type	Author / Institution	Why Watch
27 Oct 2026	RM001 in Transfusion-Dependent Beta-Thalassemia	Session 2b: (Epi)genome editing I	Hui Xu, Refrogene Medicine	Durable clinical benefit data for promoter-modified autologous HSPCs, an alternative to BCL11A-directed editing
28 Oct 2026	DNX-2440: Phase I in Recurrent Glioblastoma	Session 3c: Oncolytic therapy & cancer gene therapy	Iñaki Beasain, CIMA, University of Navarra	Preliminary Phase I data for an oncolytic adenovirus in a setting with few durable options
28 Oct 2026	Off-the-Shelf CAR T Through In Vivo Gene Delivery	Session 5: In vivo gene therapy	Christian Buchholz, Paul-Ehrlich-Institut, Langen	Anchors the congress's largest strategic shift: engineering cells inside the patient rather than in a facility
28 Oct 2026	Mutation- and Disease-Agnostic Genome Editing	Session 6: Gene editing	David Liu, Harvard University	Sets the direction for editing platforms intended to serve many mutations from a single product
29 Oct 2026	YOLT-202: Phase I/II In Vivo Base Editing in AATD	Session 7b: Genome editing II	Simon Krooss, Hannover Medical School	Clinical proof-of-concept for in vivo base editing, presented as a Fresenius Foundation Award winner
29 Oct 2026	AVB-406: AAV-miRNA MAPT Knockdown	Session 9a: CNS & sensory II	Deirdre O'Connor, AviadoBio	Intravenous AAV targeting tau, extending CNS gene therapy beyond monogenic rare disease
30 Oct 2026	ATA-200: Phase 1 Paediatric Study in LGMD-R5	Session 11a: Cardio & muscular gene therapy	Barry Byrne, Powell Gene Therapy Center	One-year safety and pharmacodynamic data in a crowded neuromuscular AAV field
30 Oct 2026	ETX101: POLARIS Phase 1/2 in SCN1A+ Dravet	Session 12a: AAVs/non integrative vectors III	Salvador Rico, Encoded Therapeutics	Interim safety and efficacy for an AAV9 therapy in a severe developmental epilepsy



Industry-Sponsored Sessions & Focus Areas – ESGCT 2026





ESGCT 2026– Competitive Landscape at a Glance (1/2)

Name	Sessions	Companies / Assets	Why It Matters
AI-guided AAV capsid & promoter design	Multiple	Dyno Therapeutics; Lir Therapeutics; Basecamp Research; WhiteLab Genomics; Sanofi (MADCAP); Apertura Gene Therapy	Capsid discovery is shifting from directed evolution to computational design; the question will be whether AI-designed capsids hold up across species and in manufacturing
In vivo CAR-T generation	Multiple	CPTx (ssDNA-tLNP); Editas Medicine; uBriGene; Cytiva; Alaya.bio; Inceptivo; Paul-Ehrlich-Institut; UCL (VLPs)	Several delivery routes are competing to engineer T cells in the patient; the question will be which achieves adequate potency and control without lymphodepletion
Programmable insertion, base & prime editing	Multiple	Beam Therapeutics (BEAM-301); YOLT-202; Addition Therapeutics (R2); CorrectSequence; Scribe Therapeutics; Arbor Biotechnologies	The field is moving past nuclease cutting toward writing and silencing; early clinical data will begin to separate the platforms
Neuromuscular AAV	Multiple	Atamyo / Genethon (ATA-200, ATA-100, GNT0004); REGENXBIO (RGX-202); Solid Biosciences (SGT-003); Benitec Biopharma (BB-301)	Multiple programmes will report in overlapping indications, making dose, durability and hepatic safety the practical points of comparison



ESGCT 2026– Competitive Landscape at a Glance (2/2)

Name	Sessions	Companies / Assets	Why It Matters
CNS & sensory gene therapy	Multiple	Encoded Therapeutics (ETX101); AviadoBio (AVB-406); Neurogene (NGN-401); Latus Bio (LTS-101, LTS-201); Voyager (VY1706); Sensorion (SENS-601)	Clinical and preclinical programmes span epilepsy, Rett syndrome, tauopathies and hearing, testing whether CNS delivery is becoming routine
Overcoming AAV seropositivity	Multiple	Genethon (IgG-degrading enzymes); Vivet Therapeutics (VTX-PID, imlifidase); Hemotune (HemoSystem DEplete); Lir and Dyno (antibody-evading capsids)	Addressing pre-existing immunity would widen eligible populations and open redosing; the question will be whether these approaches prove durable and safe
Manufacturing, CDMO & tools	Multiple	Lonza; Sartorius; Cytiva; Merck; AGC Biologics; ReiThera; Genezen; Revvity; Thermo Fisher; PackGene; Ascend Advanced Therapies	Manufacturing is the largest commercial presence at the congress; competition is centred on yield, stable cell lines and cost of goods
Non-viral vectors & RNA delivery	Multiple	Curapath; Editas Medicine; CPTx; Circio (circVec); Serif Biomedics; Neovac; PlasmidFactory (minicircle); Sleeping Beauty transposon	Non-viral platforms are positioned on redosing, payload size and simpler manufacturing; the question will be whether efficiency now matches viral delivery



Key Industry-Sponsored Sessions at ESGCT 2026 (1/4)



• Cytiva:

- Focus Areas: **Non-Viral Delivery, Solid Tumours & In Vivo CAR-T**
- Cytiva will have the broadest scientific footprint of the tools companies, **supporting both a scientific session on non-viral vectors, nanotechnology and RNA therapeutics and a lunchtime symposium on solid tumours across modalities**. Its own abstracts are set to cover ionizable lipid nanoparticle platforms for generating CAR T cells in vivo, positioning the company around the congress's fastest-moving delivery theme



• PlasmidFactory:

- Focus Areas: **Minicircle DNA & Virus-Free CAR T Manufacturing**
- PlasmidFactory **will sponsor the 'Immunotherapy and CAR T cells I' session and a lunchtime symposium on minicircle-enabled advances**, featuring Fraunhofer IZI on a virus-free clinical CAR T manufacturing protocol and Charité/Baylor on non-viral site-specific integration. The focus will be on minicircle DNA as a route around viral vector supply



Key Industry-Sponsored Sessions at ESGCT 2026 (2/4)



• Sartorius:

- Focus Areas: AAV Process Development & CDMO Partnership
- Sartorius will host a symposium bringing together developer, technology innovator and CDMO perspectives on translating AAV innovation into therapeutic products. Its abstracts are set to address chromatography-based downstream processing of cationic lipid nanoparticles, placing the company across both viral and non-viral workflows rather than in one camp



• Lonza:

- Focus Areas: T Cell Workflows & AAV Production Platforms
- Lonza's symposium is set to cover simplified serum-free T cell workflows and platform performance for AAV production in established cell lines. The focus will be on the two processes that dominate the manufacturing track, suggesting an emphasis on platform reliability and scale rather than on any single therapeutic area



Key Industry-Sponsored Sessions at ESGCT 2026 (3/4)



• Merck:

- Focus Areas: **Lentiviral & Integrative Vector Technology**
- Merck will support a scientific session on lentiviral and integrative vectors, which is expected to cover modular all-in-one vector designs for T cell strategies and in vivo lentiviral approaches in inherited disease. The company will also appear across the manufacturing poster track, indicating interest in both vector design and production



• AGC Biologics:

- Focus Areas: **Presidential Symposium Sponsorship**
- AGC Biologics will sponsor the Presidential Symposium, the congress's highest-profile session, which carries the presidential address, keynote and award ceremonies. The sponsorship is positional rather than product-specific, and points to CDMO competition for visibility with academic developers who control early-stage pipelines



Key Industry-Sponsored Sessions at ESGCT 2026 (4/4)



• Acro:

- Focus Areas: CAR T Cell Engineering
- Acro will support a scientific session on immunotherapy and CAR T cells, where discussion is expected to address receptor engineering for clinical application, modulation of T cell recognition and award-winning work on signalling optimisation. The session places a reagents supplier alongside the most competitive scientific area of the programme



• VIRALGEN:

- Focus Areas: Clinical Experience & Long-Term Follow-Up
- VIRALGEN will support an education session on what human trials are teaching the field, expected to cover long-term effects of gene therapy, protein stress and immune responses, redosing and patient access in ultra-rare disease. For a manufacturer, this choice signals interest in what happens long after the product leaves the facility



New Entrants

Asset / Company	2026 Presence
ATA-200 (Atamyo / Genethon)	Early results from an interventional Phase 1 paediatric study in LGMD-R5, reporting safety and pharmacodynamic activity at one year
AVB-406 (AviadoBio)	An intravenous AAV-miRNA achieving sustained MAPT knockdown for Alzheimer's disease and other tauopathies; supporting iPSC-neuron poster also scheduled
ETX101 (Encoded Therapeutics)	Interim results from the POLARIS Phase 1/2 clinical trials of an AAV9-based gene therapy for SCN1A+ Dravet syndrome
YOLT-202	Clinical proof-of-concept for in vivo base editing in PI*ZZ alpha-1 antitrypsin deficiency from the Phase I/II trial
RM001 (Refrogene Medicine)	Durable clinical benefits with autologous HBG1/2 promoter-modified CD34+ HSPCs for transfusion-dependent beta-thalassemia
DNX-2440	Preliminary results of a Phase I trial evaluating a DNX-2440 oncolytic adenovirus in recurrent glioblastoma
BEAM-301 (Beam Therapeutics)	Initial data from the Phase 1/2 clinical study of an in vivo base editor targeting the R83C variant in glycogen storage disease type Ia
BB-301 (Benitec Biopharma)	Cardiovascular & muscular posters (28 and 29 Oct): responses to low-dose investigational gene therapy in oculopharyngeal muscular dystrophy with dysphagia, at 12 and 24 months

AI/ML Trends & Strategic Watchpoints at ESGCT 2026





AI / Digital Health Angle at ESGCT 2026 (1/3)

- **AI is being applied to vector design rather than to patient-facing digital health**
 - ESGCT 2026 will hold a dedicated lunchtime symposium, 'AI and machine learning in gene therapy', covering computational protein design for rapid CAR generation and CRISPR-associated transposons, alongside an education talk on AI in biomedical research. The largest concentration of AI work sits in capsid and promoter design, with abstracts from Dyno Therapeutics, Lir Therapeutics, Basecamp Research and WhiteLab Genomics
- **Computational prediction is extending into safety assessment and manufacturing**
 - Scheduled sessions apply machine learning to base editor off-target prediction, CRISPR off-target detection pipelines and immunogenicity risk assessment for CAR constructs, while manufacturing posters from Cytiva, Virica Biotech and Revvity use AI for producer cell line development and small-molecule discovery. The key question will be whether computational predictions are validated well enough to support regulatory submissions
- **Key sessions include:**
 - AI and machine learning in gene therapy (lunchtime symposium)
 - EDU14 Artificial Intelligence: Opportunities and Challenges for Biomedical Research
 - INV82 AI in ATMP regulations (Regulatory & Ethics)
 - INV30 Computational protein design as strategy for rapid CAR generation



AI / Digital Health Angle at ESGCT 2026 (2/3)

- AI-guided AAV capsid engineering and multi-modal screening for targeted podocyte gene therapy
- Designing antibody-evading AAVs with an AI lab-in-the-loop approach
- AI-enabled single-shot design of an ALPL-targeting AAV capsid with cross-species CNS and muscular delivery EDEN: a metagenomic foundation model for AI-programmable gene insertion
- Synthetic promoter engineering integrating AI-guided in silico design
- ProBE-OFF: base editor off-target prediction using a mechanistic machine learning model
- AI platform for de novo mRNA sequence design for in vivo CAR-T
- Accelerating stable AAV producer cell line development through AI-driven decision intelligence

Sessions suggest AI and computational tools are increasingly being used to improve:

- Capsid design, tropism and cross-species translation
- Antibody evasion and escape from pre-existing immunity
- Cell-type-specific promoter and regulatory element design
- Editor discovery, engineering and large-payload integration
- Off-target prediction and immunogenicity risk assessment
- mRNA and CAR construct sequence design



AI / Digital Health Angle at ESGCT 2026 (3/3)

- **Regulators are beginning to address AI within the ATMP framework**
 - The Regulatory & Ethics session will include a talk on AI in ATMP regulations from the former President of the Paul-Ehrlich-Institut, alongside international regulatory harmonisation and individualised therapies in the EU. Its placement in a regulatory rather than a technical session suggests attention is turning to how computationally designed components are evidenced and assessed
- **Digital health, as distinct from AI, has limited representation in this programme**
 - As a discovery, translational and manufacturing congress, ESGCT 2026 will concentrate its AI content on vector design, editor engineering, off-target prediction and bioprocessing. Patient-facing digital health such as remote monitoring, wearables and clinical decision-support tools will have little presence, placing the AI discussion closer to R&D and manufacturing decisions than to the delivery of care



What We're Watching for Post-ESGCT 2026

- Whether **in vivo CAR-T generation** produces potency and persistence comparable to manufactured products, and which delivery route (targeted LNP, lentiviral, VLP) advances fastest
- Whether **AI-designed capsids translate across species and into manufacturing**, rather than performing well only in the screens that produced them
- Whether **in vivo base and prime editing build on early clinical proof-of-concept** from YOLT-202 and BEAM-301 toward larger datasets
- Whether **programmable insertion technologies** such as R2 retrotransposase and large serine recombinases move from preclinical demonstration into development candidates
- Whether **approaches to AAV seropositivity**, including IgG-degrading enzymes and imlifidase pre-treatment, enable redosing and widen eligible populations
- Whether **neuromuscular and CNS AAV programmes differentiate** on durability and hepatic safety as several report in overlapping indications
- Whether **manufacturing advances reduce cost of goods** sufficiently to support the access and reimbursement challenges raised in the programme
- Whether **regulatory harmonisation and EU reform discussions** translate into workable routes for ultra-rare and academically developed ATMPs

Strategic Insights and Strategy Development is our focus



COMPETITIVE STRATEGY
AND INTELLIGENCE



DIGITAL HEALTH, ARTIFICIAL
INTELLIGENCE AND IoT



GENE & CELL THERAPY



VACCINES & INFECTIOUS
DISEASES



INVESTMENT INSIGHTS

e: INFO@LQVENTURES.COM

w: WWW.LQVENTURES.COM