

ARM & GENOTHER WORKSHOP

From Platform to Patient: Defining Market Readiness for Genetic Medicines

Institut Imagine
24 Boulevard du Montparnasse
75015 Paris, France

Hybrid Format
June 19, 2026

MEETING AGENDA — JUNE 19, 2026

8:00 – 9:00

Registration & Breakfast

9:00 – 9:20

Opening Remarks

- **Sharon Anderson, Alliance for Regenerative Medicine** (10 min)
- **Marco Pintore, GenoTher** (10 min)

9:20 – 9:40

Keynote Presentation (20 min)

Industrializing Gene Therapy Platforms in Europe

This keynote will examine the current state and future trajectory of gene therapy manufacturing in Europe, exploring what it takes to transition platform technologies from academic proof-of-concept to robust, industrial-scale production capable of reliably delivering therapies to patients.

- **Frédéric Revah, CEO, Genethon**

Session 1: The Integrated Translational Ecosystem

This session explores how integrated translational ecosystems connecting academic research, clinical development, and manufacturing can accelerate the journey from discovery to patient access. Discussion will focus on successful collaboration models, the role of public-private partnerships, and strategies for bridging the gap between innovation and scalable production.

9:40 – 10:00

Presentation (20 min)

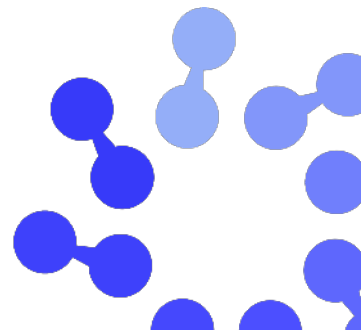
Institut Imagine: Bridging Patients, Research & Clinical Development

An inside look at how Institut Imagine's patient-centered model connects rare disease research directly to clinical programs, creating a blueprint for translational success in genetic medicine.

- **Bana Jabri, Institut Imagine**

10:00 – 10:30

Panel Discussion: Translational Ecosystems as Accelerators



Panelists will debate what makes a translational ecosystem effective, how public and private funding models can better support early-stage development, and what lessons from existing hubs in the UK, France, and Scandinavia can be applied more broadly.

Panelists:

- **Miguel Forte, Kiji Therapeutics (moderator)**
- **Thomas Carlsen, Cellerator**
- **Stephen Ward, CGT Catapult**
- **Marco Pintore, GenoTher**

10:30 – 10:45

Morning Break

Session 2: Defining Control Strategy in Platform Genetic Medicines

This session examines the analytical and quality control challenges unique to platform-based genetic medicines. Presentations and discussion will address multi-attribute potency assays, next-generation sequencing for identity and impurity profiling, and what constitutes a fit-for-purpose control strategy across the product lifecycle.

10:45 – 11:05

Presentation (20 min)

Addressing Analytical Challenges in Gene Therapy: Multi-Attribute Potency & Beyond

This presentation will explore how multi-attribute potency assays and deeper analytical characterization can strengthen the link between product quality and clinical performance for platform-manufactured gene therapies.

- Speaker, Organization

11:05 – 11:25

Presentation (20 min)

Identity, Impurities & Genome Integrity – NGS and Beyond

A deep dive into advanced characterization tools, including next-generation sequencing, for assessing vector identity, residual impurities, and genome integrity across gene therapy manufacturing campaigns.

- **Matthew Hewitt, Charles River Laboratories**

11:25 – 11:55

Panel Discussion: What Constitutes a “Sufficient” Control Strategy?

This panel will explore where the bar should be set for analytical control strategies in platform gene therapies, balancing regulatory expectations, manufacturing feasibility, and the evolving science of product characterization.

Panelists:

- Speaker, Organization

Session 3: Achieving Manufacturing Robustness Across Modalities

This session tackles the challenge of manufacturing reproducibility as gene therapy platforms scale across product variants and production sites. Discussion will focus on process characterization, technology transfer, and strategies for maintaining consistency in viral vector production at commercial scale.

11:55 – 12:15

Presentation (20 min)

Reproducibility at Scale – Platform Expansion

This presentation will address the real-world challenges of maintaining process consistency as gene therapy platforms expand to new products, production sites, and commercial-scale operations.

- **John Tomtishen, Catalent**

12:15 – 12:20

Short Q&A / Transition

12:20 – 1:20

Lunch

Session 4: Regulatory Convergence – Platform Thinking in Practice

This session addresses how regulators and industry can align on risk-based frameworks for evaluating platform-manufactured genetic medicines. Discussion will cover platform comparability approaches, the evolving regulatory landscape in Europe, and practical strategies for streamlining submissions while maintaining patient safety.

1:20 – 1:40

Regulatory Presentation (20 min)

EMA Perspective on Platform Comparability

A regulatory perspective on how the EMA is approaching platform-based development, including expectations for comparability data, platform master files, and the balance between flexibility and rigor in the ATMP regulatory framework.

- **Marcel Hoefnagel, Dutch Medicines Regulatory Agency (MEB-CBG) and Chair, EMA Quality Innovation Group**

1:40 – 2:20

Panel Discussion: Risk-Based Regulatory Frameworks

Panelists will discuss how risk-based approaches can reduce regulatory burden without compromising safety, the practical challenges of implementing platform strategies across jurisdictions, and where industry and regulators can find common ground.

Panelists:

- **Rehma Chandaria, Cell & Gene Therapy Catapult**
- **Sibylle Herzer, Kite Pharma**
- **Andrea Braun, Autolus**
- **Marcel Hoefnagel, Dutch Medicines Regulatory Agency (MEB-CBG) and Chair, EMA Quality Innovation Group**

Session 5: Market Access & Accelerating Speed to Market for ATMPs

This session focuses on the policy and commercial pathways shaping ATMP availability in Europe. Discussion will cover the EU Biotech Act's implications for speed to market, reimbursement and access models, and the role of cross-stakeholder collaboration in ensuring genetic medicines reach the patients who need them.

2:20 – 2:40

Presentation (20 min)

EU Biotech Act & Speed to Market

This presentation will examine how the EU Biotech Act and related policy initiatives are reshaping the regulatory and commercial landscape for advanced therapies, and what this means for the timeline from approval to patient access.

Presenter:

- **Caterina Giovagnoni, EC SANTE**

2:40 – 3:20

Panel Discussion: Accelerating ATMP Access in Europe

Panelists will explore the persistent barriers to ATMP market access in Europe, including reimbursement models, HTA processes, and cross-border collaboration, and discuss practical solutions for getting approved therapies to patients faster.

Panelists:

- **Adam Parnaby, BMS**
- **Ilaria Villa, Fondazione Telethon**
- **Didier Rouy, Sanofi**

3:20 – 3:35

Afternoon Break

Session 6: Technical Maturity & Capital Formation

This session explores the relationship between manufacturing readiness and investment confidence in the gene therapy sector. Discussion will address what technical and operational milestones investors look for, how manufacturing maturity de-risks clinical and commercial programs, and what it takes to attract growth-stage capital in the current environment.

3:35 – 3:50

Presentation (15 min)

Manufacturing Readiness as an Investment Signal

This presentation will explore how manufacturing maturity, including process robustness, scalability, and supply chain readiness, has become a key criterion for institutional investors evaluating gene therapy companies.

- Speaker, Organization

3:50 – 5:00

Panel Discussion: What Technical Maturity Unlocks Capital?

Panelists from the investment and biotech communities will discuss the specific technical and operational benchmarks that shift a company from early-stage risk to investable opportunity, and how the current funding environment is shaping portfolio decisions in genetic medicine.

Panelists:

- **Charles-Edouard Ecurat, Agence de l'Innovation en Santé (AIS)**
- **Sven Kili, Saisei Ventures**
- **Mathieu Losguardi, WhiteLab Genomics**

5:00 – 5:20

Closing Remarks

- **Sharon Anderson, Alliance for Regenerative Medicine**
- **Maria Grazia Biferi, GenoTher**

5:20 – 6:30

Networking Reception

Rooftop Terrace