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Gene Therapy for Rare Disorders *Europe*

31st October – 2nd November 2017, London

Overcoming Regulatory, Reimbursement, Clinical & Manufacturing Hurdles to Pioneer the Next Generation of Gene Therapies

Hear from 20+ industry leaders including:

Sven Kili
VP, Head of Cell & Gene Therapy Development
GSK

Sander van Deventer
Managing Partner, **Forbion Capital Partners** & CSO **uniQure**

Hanspeter Rottensteiner
Director, Gene Therapy
Shire

Wim Scheele
Executive Director, Rare Disease Clinical Development & Operations
Pfizer

Christiane Niederlaender
CAT Member, Senior Quality Assessor
UK Medicines and Healthcare Products Regulatory Agency (MHRA)

Jean-Philippe Combal
CEO
Vivet Therapeutics

Detlev Parow
Head, Department of Care Management Development
DAK

Aniz Girach
CMO
NightstarX

Adrien Lemoine
VP, Business Development & Operations
Orchard Therapeutics

"This is a timely venue to connect and hear from the practitioners of this technology platform on the merits as well as remaining challenges"

Seng Cheng, Global Head of Research, Rare Diseases & Gene Therapy, Sanofi-Genzyme

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www.genetherapy-europe.com

hansonwade

Your Guide to Delivering Gene Therapies to Market

Gene therapies are poised to redefine the treatment of rare diseases but these conditions present many distinct development challenges.

Gene Therapy for Rare Disorders Europe is devoted to overcoming these challenges, focusing specifically on the **clinical, manufacturing, regulatory and reimbursement** hurdles that need to be overcome for gene therapies to reach their full potential.

Uniting leaders from **Pfizer**, **GSK**, **Shire** and **uniQure**, this meeting focusses specifically on delivering practical insights into developing the next generation of gene therapies that promise **improved efficacy, enhanced safety and commercial viability.**

Join your colleagues at this uniquely focused event and discover how to enhance every stage of your gene therapy development to achieve commercial success.

"I've gained quite a lot from this conference – it's allowed us to think outside the company and get other people's impressions and experiences and that will benefit the entire area and landscape"
Sven Kili, GSK, 2017 Attendee

"It was a very successful first conference. I look forward to the next one"
Stephen Smith, Audentes Therapeutics, 2017 Attendee

Improve Every Element of Your Gene Therapy Approach

1. Shape Your Gene Therapy Strategy
Hear strategic insights into the future of the gene therapy field from Pfizer, GSK and uniQure

2. Enhance Vector Technology
Learn how to improve the efficacy and tissue targeting of gene therapy vectors for a greater therapeutic response with Vivet Therapeutics

3. Select Appropriate Endpoints
Investigate the key endpoints being explored in gene therapy trials and how the regulators view these endpoints in the ophthalmology space with NightstarX

4. Navigate the Regulatory Landscape
Explore the impact of regulatory guidelines and learn how to engage the regulators most effectively in the rare disease field through a case study from the MHRA

5. Successfully Scale Up Manufacturing
Overcome the challenges involved in transitioning gene therapy development from academic to GMP commercial scale with insights from BioMarin

6. Understand Potential Reimbursement Strategies
Investigate critical lessons that can be taken from past reimbursement strategies and how to enhance these approaches in the future, with the DAK

7. Manage the Budget Impact of High Cost Gene Therapies
Critically evaluate the relative advantages of annualisation and amortisation strategies with the Office of Health Economics

8. Gain the Investors' Perspective
Learn what investors look for and how they assess the gene therapy space, with insights from Syncona

9. Forge Mutually Beneficial Alliances
Discover how effective global collaborations can progress the field to reach its full potential with details from Orchard and TIGET

10. Standardise Cross-Industry Process Development
Improve process development practices and understand the necessity of cross-industry development standardisation with insights from Shire

Expert Speaker Faculty

Diego Ardigo
Project Leader - Advanced Therapy Medicinal Product, Biologics, Corporate Drug Development, Research & Development
Chiesi

Robert Baffi
EVP, Technical Operations
BioMarin

Alexis Cockroft
Regulatory Manager, Biopharm CMC Regulatory Affairs
GSK

Jean-Philippe Combal
CEO
Vivet Therapeutics

Emily Culme-Seymour
External Strategy Manager, Gene Therapy, Rare Diseases Unit
GSK

Joseph Earley
Senior Scientist, Downstream Process Development
Allergan

Michela Gabaldo
Head, Alliance Management & Regulatory Affairs
TIGET

Aniz Girach
CMO
NightstarX

Ann Gorman
Director, Regulatory Science
Voisin Consulting

William Green
Senior Research Consultant
York Health Economics Consortium

Grace Hampson
Senior Economist
Office of Health Economics

Reinout Hesselink
Consultant, Cell & Gene Therapy
Exmoor Pharma Consulting

Chris Hollowood
Chief Investment Officer
Syncona

Sven Kili
VP, Head of Cell & Gene Therapy Development
GSK

Magda Papadaki
Head, Manufacturing Innovation
The Association of the British Pharmaceutical Industry (ABPI)

Hanspeter Rottensteiner
Director, Gene Therapy
Shire

Adrien Lemoine
VP, Business Development & Operations
Orchard Therapeutics

Gopalan Narayanan
VP, Disruptive Biologics
Voisin Consulting

Christiane Niederlaender
CAT Member, Senior Quality Assessor
UK Medicines and Healthcare Products Regulatory Agency (MHRA)

Samantha Parker
SVP, Chief Patient Access Officer
Lysogene

Detlev Parow
Head, Department of Care Management Development
DAK

Wim Scheele
Executive Director, Rare Disease Clinical Development & Operations
Pfizer

Rafaele Tordjman
Independent Venture Capitalist, Independent board member, Obseva Founder & Chairwoman, **WITH Association**

Pamela Tranter
Head, Translational Research Group
UCL

Sander van Deventer
Managing Partner, **Forbion Capital Partners**
CSO
uniQure

"I really enjoyed the broad spectrum of topics that the conference covered"

Samantha Galuska, Sanofi, 2017 Attendee

Conference Day One | Wednesday 1st November, 2017

7.30 Registration, Coffee & Networking

Sander van Deventer, Managing Partner, **Forbion Capital Partners** & CSO, **uniQure**

8.20 Chair's Opening Remarks

Sander van Deventer, Managing Partner, **Forbion Capital Partners** & CSO, **uniQure**

8.30 Keynote: Investigating Critical Challenges in the Gene Therapy Field – Mapping out the Landscape & Discussing Future Strategies

- Understanding factors contributing to the clinical efficacy of gene therapies and investigating strategies to enhance this efficacy
- Outlining the case for different reimbursement models and discussing potential future approaches
- Improving the safety of gene therapies and investigating novel technologies and platforms to reduce immunogenicity while maintaining efficacy

Sven Kili, **GSK**

Wim Scheele, **Pfizer**

Hanspeter Rottensteiner, **Shire**

9.00 Keynote Panel Discussion: The Future of Gene Therapies for Rare Disease

After decades of unfulfilled potential, gene therapies are gaining momentum. Clearly a significant amount of progress has been made in this field in Europe, but how can we ensure that future gene therapies do not encounter the same commercial hurdles that have been experienced in the past?

- Assessing the unique considerations for development in the rare and ultra-rare disease space – how can companies meet these demands?
- Bridging the gap between academia and early proof-of-concept studies and commercialisation
- Considering approaches to create value in clinical programs from the early stages

Jean-Philippe Combal, CEO, **Vivet Therapeutics**

10.00 Investigating Novel Vector Technology with the Promise of Enhancing the Efficacy of Gene Therapy

- Learning about the limitations of current vector technology – where can improvements be made regarding expected benefits?
- Strategies to enhance the tissue targeting of gene therapies of *in vivo* AAV– how to deliver a greater quantity of therapeutic to a specific tissue
- Minimising the complications associated with safety and potential re-dosing by examining novel delivery technologies

10.30 Speed Networking & Morning Refreshments

Enhancing the Speed & Robustness of Clinical Development

Aniz Girach, CMO, **NightstarX**

11.30 The Selection of Endpoints for Ophthalmology Gene Therapy Trials in the Rare Disease Space

- What are the key endpoints being explored at the moment and in the future?
- What endpoints are realistically measurable in the timescale?
- Investigating the regulators' feedback relating to these endpoints

Wim Scheele, Executive Director, Rare Disease Clinical Development & Operations, **Pfizer**

12.00 Defining & Maintaining Clinical Standards – Is Gene Therapy a Special Case?

- Examining the protocols and clinical practices that need to be in place to avert safety concerns in gene therapy trials
- Understanding per-patient verification and requirements for clinical development
- Setting the standard – ensuring that ICH guidelines are adhered to correctly throughout the gene therapy field

12.30 Lunch & Networking

1.30 Interactive Roundtable Discussions: Drilling into Key Areas of the Gene Therapy Space

Drive your own learning and crowd-source ideas and discover multiple perspectives on the key issues affecting your development efforts in the gene therapy space. Join roundtable discussions that have been specifically designed to enable you to leave with insights that you can immediately implement into your own drug development programs.

Following discussions in the intimate and open roundtable format, the table leaders will form a panel to feed back to the entire audience on the key topics that were discussed in the session.

Form a comprehensive understanding of the gene therapy development and commercialisation process and the strategies that are employed to overcome challenges at every point along the way.

2.30 Afternoon Refreshments & Networking

Navigating the Complex Regulatory Landscape

Christiane Niederlaender, CAT Member, Senior Quality Assessor, **UK Medicines & Healthcare Products Regulatory Agency (MHRA)**

3.00 MHRA Perspective on Gene Therapies for Rare Disorders

- Learning what the regulators are looking for in this field
- Understanding how to engage the regulators most effectively at critical time points in the development of gene therapy products
- How regulatory guidelines impact on every stage of the gene therapy development process

Alexis Cockroft Regulatory Manager, Biopharm CMC Regulatory Affairs **GSK**

3.30 Registering a Cell-Based Gene Therapy Product - CMC Considerations

- Understanding the guidance, legislation and other regulatory requirements you should consider
- Overcoming practical CMC challenges for autologous cell-based products
- Sharing CMC lessons learnt from the Strimvelis MAA

Michela Gabaldo, **TIGET**

Christiane Niederlaender, **UK Medicines & Healthcare Products Regulatory Agency (MHRA)**

Gopalan Narayanan, **Voisin Consulting**

Magda Papadaki **The Association of the British Pharmaceutical Industry (ABPI)**

4.00 Investigating the Regulatory Perspective on Clinical Development, Manufacturing & Market Approval

- Understanding the variations in clinical trial application of regulations across Europe
- What do you need to demonstrate in order to gain accelerated approval?
- Learning about the benefits and drawbacks of conducting commercial reviews with the regulators compared to national health bodies in various EU countries

Sander van Deventer, Managing Partner, **Forbion Capital Partners** & CSO **uniQure**

4.45 Chair's Closing Remarks

5.00 End of Conference Day One

Conference Day Two | Thursday, 2nd November, 2017

Sander van Deventer, Managing Partner, **Forbion Capital Partners** & CSO, **uniQure**

8.20 Chair's Opening Remarks

Discussing Critical Factors Influencing Potential Reimbursement Approaches

Sven Kili, VP, Head of Cell & Gene Therapy Development, **GSK**

8.30 Keynote: Establishing Value for Gene Therapy Products by Implementing Effective Reimbursement Strategies

- Considering the patient and carers with broader implications, not just the therapy in reimbursement planning
- Investigating new reimbursement models for gene therapy - strengths and weaknesses
- Learning first hand from experiences in pricing Strimvelis in the European landscape

Detlev Parow, Head, Department of Care Management Development, **DAK**

9.00 The Payer Perspective: Glybera Insights – What are the Key Takeaways?

- Pricing of gene therapy products – is there a ceiling price and how can this be identified?
- Assessing three different gene therapeutic approaches – key factors to consider e.g. treatment setting and regulatory framework
- Evaluating the evidence required with limited patient populations in the rare disease space

Grace Hampson, Senior Economist, **Office of Health Economics**

9.30 Options for Managing Budget Impact of High Cost Gene Therapies

- Understanding the challenges of reimbursement for high cost gene therapies
- Investigating different approaches to manage risk – discussing the relative benefits and drawbacks of annualisation and amortisation strategies

Detlev Parow, **DAK**

William Green, **University of York**

Sven Kili, **GSK**

Diego Ardigo, **Chiesi**

10.00 Multi-Stakeholder Panel on Executing Effective Pricing, Reimbursement & Market Access Approaches

- Recognising how payers view value delivered and discussing what they are prepared to pay for
- Identifying the full impacts of the therapy to ensure the full costs of the disease are recognised – such as hospital costs, ancillary costs, transport, impact on other family members – rather than simply the costs of the medicine
- Considering the use and setting of the therapeutic and how this will impact on reimbursement

10.30 Morning Refreshments & Networking

Enhancing Process Development & Manufacturing to Prepare for Commercialisation

Hanspeter Rottensteiner, Director, Gene Therapy, **Shire**

11.00 Optimising Vectorology, Process Development & Cross-Industry Standardisation

- Establishing effective vector optimisation programs
- Improving interactions between research, nonclinical development and process development teams
- Discussing the necessity of cross-industry standardisation of development efforts in this space

Joseph Earley, Senior Scientist, Downstream Process Development, **Allergan**

11.30 Enhancing the Manufacturability of Gene Therapies

- Understanding the key challenges and issues to consider prior to scale-up
- Evaluating the equipment requirements and how the manufacture of viruses contrasts with other fields
- Investigating strategies to bypass the requirement of ultracentrifuges


Robert Baffi, EVP, Technical Operations, **BioMarin**

12.00 **Improving the Scalability of Gene Therapy Manufacturing**

- Overcoming challenges encountered in transitioning from lab scale to late-stage GMP commercial scale manufacturing
- Examining the impact of vector choice on scalability – comparing serotypes and overcoming the challenges experienced in the scale up of self-complementary vectors
- Controlling the cost of goods throughout scale up to guarantee cost-effectiveness

12.30 **Lunch & Networking**

Investigating the Funding Opportunities Available in Europe


Pamela Tranter, Head, Translational Research Group, **UCL**

1.30 **Identifying Opportunities to Translate Gene Therapy in Academia & Key Factors to Address Using Case Study Examples**

- Understanding the de-risking and evidence required to support the commercial route forward
- Analysing the capacity, capability and cost issues related to gene therapy development
- Learning about the funding opportunities available in this field


Chris Hollowood, **Syncona**


Rafaele Tordjman, **Independent Venture Capitalist**


Sander van Deventer, **Forbion Capital Partners**, **uniQure**

2.00 **Panel Discussion: How do Investors View the Gene Therapy Space?**

- How flexible are VCs in this space?
- Do investors look for a focussed or broad pipeline when evaluating gene therapy companies?
- Contrasting the funding scene in Europe compared to the US
- Do VCs view gene therapy companies as platforms which can be adapted based on changes in gene of interest or project-based investments?
- Understanding the scientific criteria investors use to determine funding decisions

2.30 **Afternoon Refreshments & Networking**

Strengthening Collaborations Between Academia & Industry


Samantha Parker, SVP, Chief Patient Access Officer, **Lysogene**

3.00 **Uniting the Rare Disease Community Around Key Patient Affairs and Policy Objectives**

- Understanding the specific and unique characteristics of the rare disease space
- Learning how to best involve patients, industry leaders and regulators to map out policies and guidelines in this area
- Sharing case studies investigating Lysogene's work in this space


Adrien Lemoine, **Orchard Therapeutics**


Emily Culme-Seymour, **GSK**


Michela Gabaldo, **TIGET**


Magda Papadaki **The Association of the British Pharmaceutical Industry (ABPI)**

3.30 **Panel Discussion: How can Organisations Forge Mutually Beneficial Alliances in the Gene Therapy Field?**

- Evaluating the nature of deal structures and collaborations between biopharma companies, academic institutions and solution provider companies– how have they evolved and how can they be improved?
- How can organisations in the field collaborate more effectively to bring therapies to patients more rapidly?


Sander van Deventer, Managing Partner, **Forbion Capital Partners** & CSO, **uniQure**

4.15 **Chair's Closing Remarks**

4.30 **End of Conference**

Pre-Conference Workshop Day | Tuesday, 31st October

Workshop A: 9:00am - 12:00pm

Investigating the Unique Regulatory Landscape of the Gene Therapy Field

As the gene therapy space advances and clinical investigations continue to produce promising results, uncertainty about the route to successful regulatory approval and commercialisation remains a significant challenge.

Attend this workshop to gain an in-depth understanding of key regulatory issues for gene therapies for both clinical development and marketing authorization at this pivotal time for the gene therapy field.

Attendees will learn about:

- CMC issues related to the development of gene therapies
- Impact of environmental risk assessments on clinical trials applications for GMO-based medicinal products
- Specific regulatory requirements for gene therapies for successful MAAs

Workshop Leader:
Ann Gorman
Director, Regulatory Science
Voisin Consulting

Workshop B: 1:00pm - 4:00pm

Unlocking the CMO Selection Process: How to Select & Manage External Manufacturers Effectively

Identifying CMOs with sufficient capacity, expertise and facilities to handle outsourced projects in cell and gene therapy is a major challenge in Europe and globally.

Investigate the balance between internal and external manufacturing and learn how to work effectively with external partners who will be able to assist with the specific demands of your gene therapy programs

As scale-up and commercialisation of gene therapy programs draws closer, this workshop session is your opportunity to get up to speed with the most successful outsourcing strategies in this field.

Attendees will learn about:

- The criteria you should be using to adequately assess potential partners to outsource projects to in the gene therapy space
- How to tailor and assess the specific process requirements of an external manufacturer
- Which kind of tools to use for data-driven CMO selection
- Key factors impacting the decision to develop in-house capabilities or outsource services to third parties

Workshop Leader:
Reinout Hesselink
Consultant, Cell & Gene Therapy
Exmoor Pharma Consulting

PARTNER WITH US

Gene Therapy for Rare Disorders Europe is your fastest route to organisations prioritizing gene therapy development.

This meeting will bring together decision makers from pharma and biotech companies who are committed to speeding up the development of their gene therapy programmes.

This is your opportunity to influence industry thinking as the landscape continues to develop and **capitalise on emerging areas where your solution can impact progress.**

Partner with this meeting to ensure you are front of mind as decisions on strategy are being made for the next 12 months.

Get in touch today for more information about the various partnership opportunities available.

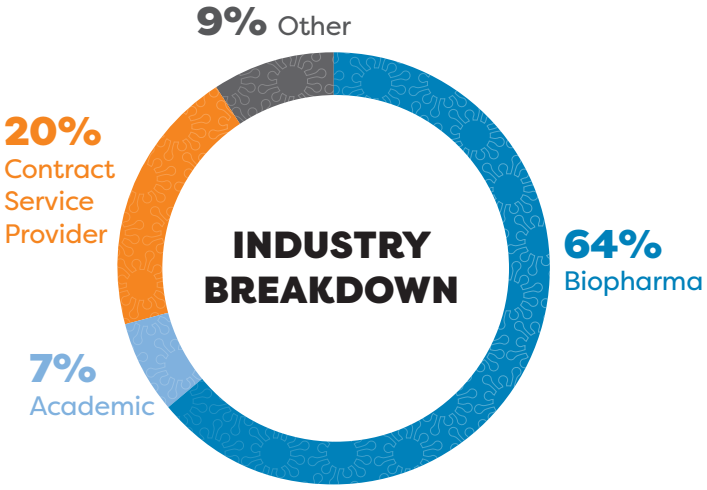
PREVIOUS ATTENDEES

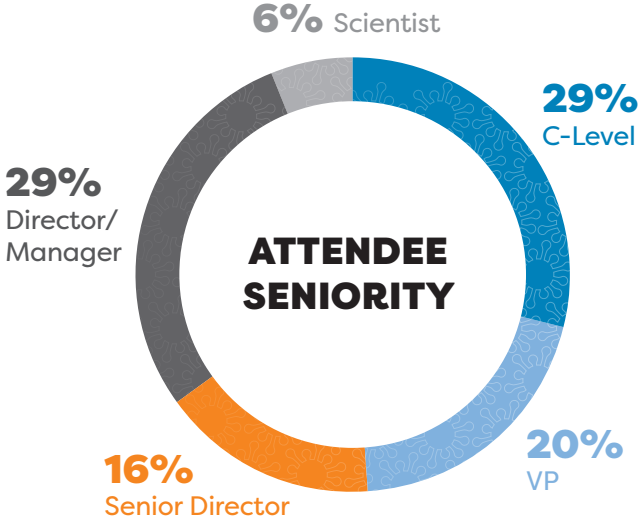
Abeona Therapeutics, agtc, Audentes Therapeutics, Avexis, AVROBIO, Bayer HealthCare Pharmaceuticals, BIOMARIN, bluebirdbio, Dimension Therapeutics, European Medicines Agency, FDA, gsk, Orchard therapeutics, Pfizer, Bayer HealthCare, Shire, Spark Therapeutics, uniQure

"This conference is a good opportunity for a lot of the industrial leaders to be able to come together and learn how different companies will tackle challenges from different perspectives, and it allows us to learn from each other"

Vivian Choi, Associate Director, Head of Gene Therapy Research, Shire Pharmaceuticals

ATTENDEE BREAKDOWN





* Statistics based on Gene Therapy for Rare Disorders Boston, April 2017

BECOME A PARTNER

Tim Green
Business Development Manager
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READY TO REGISTER?

3 EASY WAYS TO BOOK

www.genetherapy-europe.com/register

Tel: +44 203 141 8700

Email: register@hansonwade.com

1. Engage directly with industry leaders from biotech, pharma, payers and the regulatory authorities in an intimate environment
2. Understand the clinical, regulatory, reimbursement and manufacturing steps that need to be taken to realize the commercial success of gene therapies globally
3. Immerse yourself in 3 days of in-depth talks and interactive sessions focusing specifically on gene therapies for rare disease

Team Discounts*

- **10% discount – 3 delegates**
- **15% discount – 4 delegates**
- **20% discount – 5 or more delegates**

Please note that discounts are only valid when three or more delegates from one company book and pay at the same time.

SECURE YOUR PLACE

INDUSTRY PRICING	Register & Pay before Friday September 22, 2017	Standard Price
Gold: Conference & 2 Workshops	£2797 + VAT* **(save £400)**	£2997 + VAT* **(save £200)**
Silver: Conference & 1 Workshop	£2298 + VAT* **(save £300)**	£2498 + VAT* **(save £100)**
Bronze: Conference Only	£1799 + VAT* **(save £200)**	£1999 + VAT*
Workshops - Individual	£599 + VAT*	

ACADEMIC & NOT-FOR-PROFIT PRICING	Register & Pay before Friday September 22, 2017	Standard Price
Gold: Conference & 2 Workshops	£1677 + VAT* **(save £240)**	£1797 + VAT* **(save £120)**
Silver: Conference & 1 Workshop	£1378 + VAT* **(save £180)**	£1498 + VAT* **(save £60)**
Bronze: Conference Only	£1079 + VAT* **(save £120)**	£1199 + VAT*
Workshops - Individual	£359 + VAT*	

*VAT at 20%

VENUE

De Vere Grand Connaught Rooms
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London, WC2B 5DA

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TERMS & CONDITIONS

Full payment is due on registration. Cancellation and Substitution Policy: Cancellations must be received in writing. If the cancellation is received more than 14 days before the conference attendees will receive a full credit to a future conference. Cancellations received 14 days or less (including the fourteenth day) prior to the conference will be liable for the full fee. A substitution from the same organization can be made at any time.

Changes to Conference & Agenda: Hanson Wade reserves the right to postpone or cancel an event, to change the location or alter the advertised speakers. Hanson Wade is not responsible for any loss or damage or costs incurred as a result of substitution, alteration, postponement or cancellation of an event for any reason and including causes beyond its control including without limitation, acts of God, natural disasters, sabotage, accident, trade or industrial disputes, terrorism or hostilities.

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