BOOK NOW AND SAVE UP TO £400

2nd Annual Gene Therapy for Rare Disorders *Europe*

22-24 October | London, UK

Dedicated to Realising the Commercial Potential of Gene Therapies

Hear from 20+ industry leaders including:

Steven Pearson
Founder & President
ICER

Hai Zhang
Director, Pharma M&A
Novartis

Alan Griffith
MSAT Manager
MeiraGTx

Matthew Hankinson
Associate Director,
Analytical Development
Allergan

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

Jeff Sevigny
CMO
Prevail Therapeutics

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

Detlev Parow
Head, Pharmaceuticals
Department
DAK-Gesundheit

Karen Aiach
Founder & CEO
Lysogene

"This is "the conference" to attend for professionals that are already involved or about to embark in the gene therapy space."

Bill White, Senior Director, Market Access, AveXis

Tel: +44 (0)20 3141 8700 **Email:** info@hansonwade.com Gene Therapy for Rare Disorders
www.genetherapy-europe.com

hansonwade

Maximise the Commercial Potential of Gene Therapies

2018 is the year gene therapies come into their own. After decades on unfulfilled potential, this space is gaining tangible momentum, particularly in addressing the significant unmet need in a range of rare diseases.

Gene Therapy for Rare Disorders Europe will unite industry leaders to discuss the late-stage challenges that need to be overcome to deliver more gene therapies to market.

Rather than early stage basic science, this meeting is solely devoted to addressing the latest clinical, manufacturing, regulatory and commercialization challenges facing this rapidly evolving field.

This is a comprehensive guide to define your commercial path forward.

> Speakers were very well informed and relayed their experiences in understandable manner. I took away many points and topics for consideration
>
> **Kenneth Miller, Global Head, Regulatory Affairs, New Business Development, AbbVie**

> The conference covered topics across nonclinical, clinical, and manufacturing allowing a vivid journey through the paradigm of innovations in gene therapy within a short span of time
>
> **Jaya Vatsyayan, Associate Director, Shire**

Improve Every Element of Your Gene Therapy Approach

1.

In-Depth Discussions on Potential Reimbursement & Pricing Approaches

Learn first-hand about experiences in evaluating the pricing strategy for Luxterna from **ICER**, as well as insights into the European reimbursement landscape from **DAK**

2.

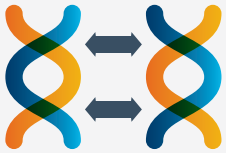
Understand Partnerships & Acquisitions in the Field

Gain insights into the fundamental aspects of effective partnerships, as well as the key aspects large pharma use to assess innovative biotech companies ahead of potential acquisitions, with insights from **Novartis** and **GSK**

3.

Navigate the Complex European Regulatory Landscape

Save time and streamline resources by understanding how **BioMarin** and **NightstaRx** approach GMO applications, manage differences in cross-border legislation and engage the regulators effectively



4.

Enhance Manufacturing, Characterisation & Analysis

Establish robust and scalable manufacturing processes and handle analytical challenges unique to the gene therapy space, with insights from **Allergan** and **MeiraGTx**

5.

Pioneer Gene Therapies in More Common Disease Indications

Understand the rationale behind **Prevail Therapeutics**' choice of gene therapy indications, as well as the challenges **CombiGene** are facing in preparing to transition to more common epilepsy indications

Expert Speaker Faculty

Karen Aiach
Founder & CEO
Lysogene

Celine Bouquet
Preclinical Manager
GenSight Biologics

Emily Culme-Seymour
Clinical Development Scientist, Rare Diseases
GSK

Omar Dabbous
VP, Global Health Economics & Outcomes Research
AveXis

Anne Douar
CDO
Vivet Therapeutics

Alan Griffith
MSAT Manager
MeiraGTx

Matthew Hankinson
Associate Director, Analytical Development
Allergan

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

Nerissa Kreher
CMO
Avrobio

Aurelie Lambert
Manager, Regulatory Affairs EU
BioMarin

Christine Le Bec
Head of CMC Analytics
Genethon

Robert Morgan
Executive Director, Regulatory Affairs
Nightstar Therapeutics

Jan Nilsson
CEO
CombiGene

Detlev Parow
Head, Pharmaceuticals Department
DAK-Gesundheit

Steven Pearson
Founder & President
ICER

Markus Peters
CCO
Agilis Biotherapeutics

Dominic Schmidt
Partner
Syncona

Jeff Sevigny
CMO
Prevail Therapeutics

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

Samantha Vieira
Senior Director, Programme Management
Nightstar Therapeutics

Hai Zhang
Director, Pharma M&A
Novartis

Isabelle Pengué Koyi
VP, Regulatory Affairs & Quality
GenSight Biologics

Hanspeter Rottensteiner
Director, Drug Discovery Gene Therapy
Shire

"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**

Conference Day One | Tuesday 23 October, 2018

7.30 **Registration, Coffee & Networking**

Sven Kili
GSK

8.20 **Chair's Opening Remarks**

Establishing Value for Gene Therapy Products: Discussing Reimbursement, Market Access & Pricing Considerations

Steven Pearson
Founder & President
ICER

8.30 **How Will Value Frameworks and Budget Impact Analysis be Used to Evaluate Gene Therapies?**

- Understanding the challenges of reimbursement for high cost gene therapies
- Balancing near-term budget impact with long-term value: what mechanisms are in place to evaluate this balance?
- Investigating various approaches to manage risk – discussing the relative benefits and drawbacks of annualisation and amortisation strategies

Detlev Parow
Head, Pharmaceuticals Department
DAK-Gesundheit

9.00 **The Payer Perspective: Investigating how Gene Therapies Require a Fundamental Change in Healthcare Delivery**

- Contrasting gene therapies with existing therapeutics – what are the key differences and how will European healthcare systems need to adapt?
- Understanding the key aspects involved in the establishment of treatment centres
- Is this a sustainable model and what are the alternatives?

Omar Dabbous
AveXis

Detlev Parow
DAK-Gesundheit

Steven Pearson
ICER

9.30 **Panel Discussion: Evaluating Value-Based Pricing Models for Gene Therapy Products**

- Investigating real-world learnings from European pricing models to incorporate into program designs
- Beyond the overview – examining at what threshold the budget impact is it even useful or meaningful examine payment over time and performance based models rather than a one-off payment
- Investigating the challenges in reducing payment models to practice, in terms of:
  - Data collection
  - Patient tracking
  - US versus EU experiences
  - Single payer versus multi payer systems

10.00 **Speed Networking & Morning Refreshments**

A Call to Arms for Standardisation

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

11.15 **Establishing Effective Manufacturing & Regulatory Standards**

- Defining the elements of gene therapy development that need to be standardised
- Investigating strategies to overcome analytical limitations to make comparability easier
- Establishing effective comparability practices
- Call to arms for regenerative medicine community

Defining & Executing Robust Clincal Development Strategies

Nerissa Kreher
CMO
Avrobio

11.30 **Incorporating Patient Input & Patient Reported Outcome Measures: A Fabry Clinical Trial Example**

- Considerations for use of PRO Measures in clinical trials
- Example of utilising a PRO Measure designed for one disease in another disease
- Collaboration with patient groups regarding PRO Measures

Celine Bouquet
Preclinical Manager
GenSight Biologics

12.00 **Supporting Clinical Development in the Gene Therapy Space Through Biodistribution & Immunogenicity Assessment**

- Understanding immune responses against gene therapy vectors
- Insights into the role of non-clinical bioanalysis in supporting the development of gene therapies
- Incorporating a regulatory perspective - understanding key early interactions with regulatory authorities

12.30 Lunch & Networking

Investigating the Funding Opportunities Available in Europe

Dominic Schmidt
Syncona

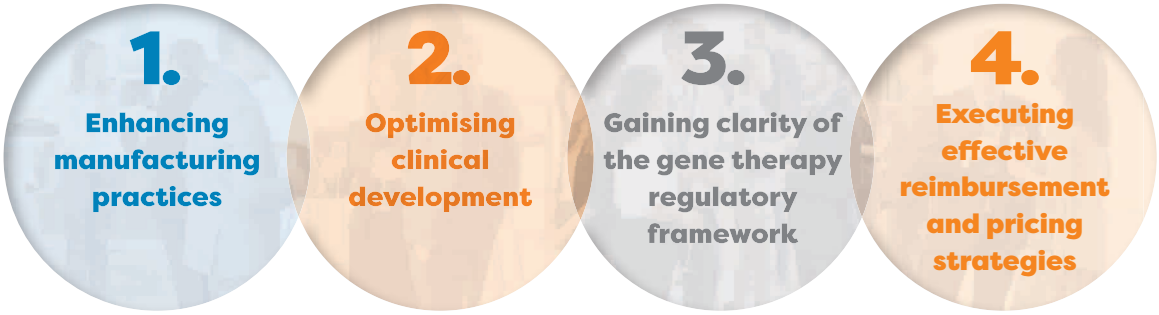
Sander van Deventer
uniQure Forbion Capital Partners

2.00 Panel Discussion: Gaining Insights into How Investors View the Gene Therapy Space

- Contrasting the funding scenes in Europe compared to the US
- What do VCs like to see and what resonates with them in their investment strategy or do they appraise programs on an individual basis?
- Understanding whether investors see gene therapies as a platform play or more project based
- How flexible are VCs and do they look for a focused or broad pipeline?

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

- Drive your own learning, 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.

3.30 Afternoon Refreshments & Networking

Establishing Mutually Beneficial Partnerships to Achieve Commercial Potential

Markus Peters
Agilis Biotherapeutics

Samantha Vieira
Nightstar Therapeutics

4.00 Panel Discussion: Understanding Unique Challenges Involved in Developing Gene Therapies in a Biotech Environment

- Understanding how gene therapy biotechs can work effectively with academic institutions to drive progress
- How can small companies without a global reach approach issues like patient recruitment across the world and cross-border legislation?
- Do companies need to change their hiring strategies to deal with the requirements of the gene therapy space?
- What are the new types of infrastructures required?

Hai Zhang
Novartis

Emily Culme-Seymour
GSK

4.30 Panel Discussion: Strategic Partnerships to Achieve Commercialisation: What are the Trends & What Does the Future Look Like?

- Gaining strategic insights from recent partnering and acquisitions in the field
- Understanding how pharma and biotech companies can work effectively together to develop gene therapies on a global scale
- Pros and cons of going it alone versus partnering
- Investigating the value that needs to be demonstrated to attract interest from larger entities

Sven Kili
GSK

5.00 Chair's Closing Remarks

5.15 End of Conference Day One

Conference Day Two | Wednesday 24 October 2018

8.20 Chair's Opening Remarks

Supporting Pioneering Gene Therapy Development by Enhancing Manufacturability, Dose Selection & Adding to the Analytical Toolbox

Alan Griffith
MSAT Manager
MeiraGTx

8.30 Scaling Manufacturing to Deal with the Demands of Commercial Gene Therapy Products

- Establishing robust and scalable manufacturing processes early in development to support larger scale manufacturing in state-of-the-art facilities
- Investigating novel manufacturing technologies to improve efficiency at scale
- Understanding the role of automation in gene therapy manufacturing

Hanspeter Rottensteiner
Director, Drug Discovery Gene Therapy
Shire

9.00 Navigating the Capacity Bottleneck – Contrasting In-House and External Manufacturing Options & Understanding the Shire Approach

- Evaluating the long-term strategic impacts of internal vs external manufacturing decisions
- Case studies outlining the various advantages and disadvantages of internal and external manufacturing
- Complimenting drug development activities with in-house manufacturing to drive forward the gene therapy field as a whole

Matthew Hankinson
Associate Director, Analytical Development
Allergan

9.30 Developing Effective Process Characterisation & Analytical Tools

- Improving the analytical tool box: Understanding the most effective analytical approaches for gene therapy products
- Understanding analytical challenges specific to the rare disease space
- Refining processes to deal with low concentrations

10.00 Morning Refreshments & Networking

Anne Douar
CDO
Vivet Therapeutics

11.00 Finding the Right Dose From Mice to Human: A Complex Equation

- Complexity of first in human with therapeutic objectives versus safety
- How to calculate the dose and translation from mice to monkey and then human
- Impact of pre-existing immunity?
- Perspectives for re-dosing?
- Regulatory perspectives

Investigating the Indications that Will Allow Gene Therapies to Achieve their Full Potential

Jan Nilsson
CombiGene

Jeff Sevigny
Prevail Therapeutics

Alan Griffith
MeiraGTx

11.30 Panel Discussion: The Next Step – Pioneering Gene Therapies in More Common Diseases

- Understanding the challenges involved in transitioning from rare to more common disease indications
- Will we soon run out of economically viable monogenic diseases to treat with gene therapy?
- Highlighting the incentives to build gene therapy platforms in rare indications but looking towards the future treatment of more common diseases
- Investigating how gene therapies would potentially compete with small molecule and antibody approaches in more mainstream indications

12.15 **Lunch & Networking**

Investigating European Biotechs Pioneering Gene Therapy Programs to Address Critical Unmet Medical Needs

Samantha Vieira
Senior Director, Programme Management
Nightstar Therapeutics

1.15 **Developing & Commercializing Novel One-Time Treatments for Patients Suffering from Rare Inherited Retinal Diseases that would Otherwise Progress To Blindness – the Nightstar Approach**

- Nightstar is gene therapy company solely focused on treating inherited retinal diseases that would otherwise lead to blindness
- Lead clinical candidate, NSR-REP1 being studied in Phase 3 registrational trial for Choroideremia with one year data expected in 2020
- Second clinical candidate, NSR-RPGR, being studied in Phase 1/2 trial with preliminary dose escalation data expected Q3'2018

Karen Aiach
Founder & CEO
Lysogene

1.45 **Presentation Details To Be Confirmed**

Navigating the Complex European Regulatory Landscape

Robert Morgan
Executive Director, Regulatory Affairs
Nightstar Therapeutics

2.15 **Engaging European Regulators Around Key Issues Throughout the Gene Therapy Development Process**

- Importance of partnering with regulators throughout the development pathway for gene therapy products
- Understanding how and when to engage the Authorities most effectively, with objective of agreeing a harmonised global approach
- How regulatory guidelines impact throughout the development pathway

2.45 **Afternoon Refreshments & Networking**

Aurelie Lambert
Manager, Regulatory Affairs EU
BioMarin

3.15 **Tackling the Additional Complexities of GMO Applications to Save Crucial Development Time**

- Investigating differences in GMO applications in different member states
- Understanding what can be done in parallel and what can be done sequentially
- Broadening initial GMO submissions to expand beyond small-scale phase 1 studies into later stages of development
- Navigating cross-border legislation and differences in regulatory landscape across Europe and tapping into local knowledge to select the best locations for trials

Isabelle Pengué Koyi
VP, Regulatory Affairs & Quality
GenSight Biologics

3.45 **Managing Late-Stage Gene Therapy Development in Preparation for MA/BLA Applications**

- Contrasting late-stage regulatory approaches to consider in the US vs European landscapes
- Analysing features unique to the rare disease space
- Integrating manufacturing and clinical development in regulatory strategies

4.15 **Chair's Closing Remarks**

4.30 **End of Conference**

"The conference covered topics across nonclinical, clinical, and manufacturing allowing a vivid journey through the paradigm of innovations in gene therapy within a short span of time"

Jaya Vatsyayan, Associate Director, **Shire**

Pre-Conference Workshop Day | Monday 22 October

Workshop A: 1:00pm - 4:00pm

Overcoming Analytical Challenges Encountered Throughout the Development of Gene Therapies

As an increasing number of gene therapy products progress into the advanced stages of development, the role of analytical testing is becoming increasingly crucial. These tests are invaluable in clarifying the quality of products and mitigating against unexpected delays at every stage of this development journey.

Join us to learn about:

- The unique analytical challenges posed by the complexity of gene therapy products
- Quality attributes that need to be closely monitored throughout gene therapy drug development, including potency, purity, safety, stability and efficacy
- The design of analytical assays in the context of the current EU regulatory framework

Workshop Leader:
Christine Le Bec
Head of CMC Analytics
Genethon

"Great conference, especially for one that's only been running a couple of years. All the leading companies were represented which was great to see and have a chance to network"

Matthew Hankinson, Associate Director, Analytical Development

"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

"I must say I've gained quite a lot from this Gene Therapy for Rare Disorders 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. I look forward to many more of these meetings in the future"

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

PARTNER WITH US

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

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

As the landscape continues to develop, this is your opportunity to influence industry thinking, and capitalise on emerging areas where your solution can impact progress.

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

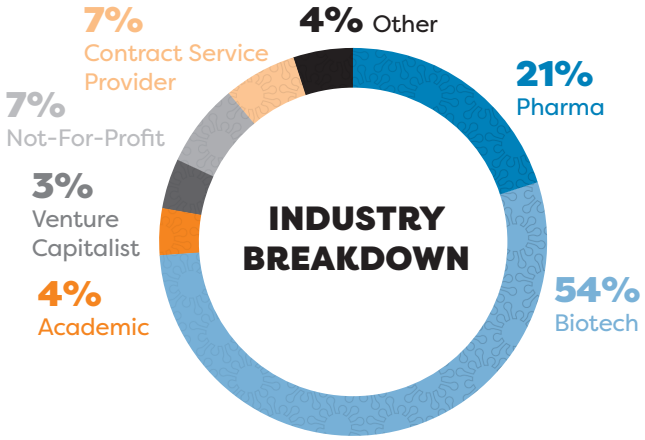
Get in touch today for more information about the various partnership opportunities available.

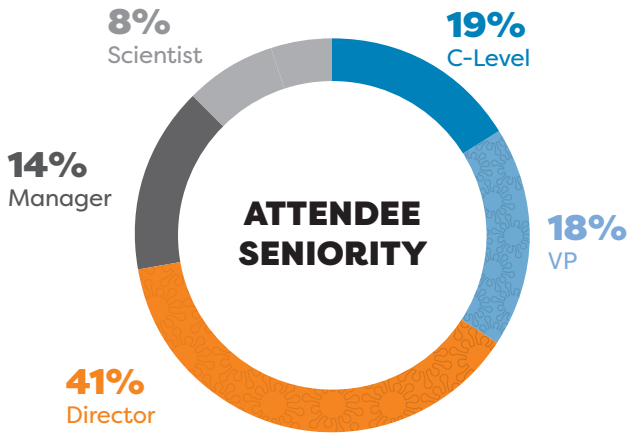
> The quality of attendees are what make a great conference and the people attending this meeting were absolutely first-rate
>
> **Adith Venkiteshwaran, New Product Strategy & Pipeline Commercialization, Rare Genetic Diseases, Sanofi Genzyme**

> It was a productive meeting with lots of networking opportunities
>
> **Syneos Health - Previous Gene Therapy for Rare Disorders Partner**

> This was a good deep dive into the world of gene therapies for rare diseases
>
> **Brammer Bio - Previous Gene Therapy for Rare Disorders Partner**

ATTENDEE BREAKDOWN





* Statistics based on Gene Therapy for Rare Disorders US 2018

BECOME A PARTNER

Rob Keast
Partnership 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 investors 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 in Europe
3. Immerse yourself in 3 days of in-depth talks and interactive sessions focusing specifically on enhancing the development of gene therapies in the rare disease space

Team Discounts

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

PACKAGE DETAILS	Register & Pay By Friday 13 July	Standard Price
Silver: Conference & 1 Workshop	£2,298 + VAT **(save £400)**	£2,598 + VAT **(save £100)**
Bronze: Conference Only	£1,799 + VAT **(save £300)**	£2099 + VAT
Workshops - Individual	£599 + VAT	

"This is the place to go to if you want to connect to experts in the field of gene therapy"

Hanspeter Rottensteiner, Director, Drug Discovery Gene Therapy, Shire

* Please note that VAT will be charged at 20%. Email info@hansonwade.com for full T&Cs.

*Academics are entitled to 40% off the drug developer prices. Use code "ACA" when registering online.

VENUE

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www.novotellondonwest.co.uk