RUNNING DIGITALLY FOR 2021

4th Annual

Gene Therapy for Rare Disorders

February 22-25, 2021 | 8-6.30 EST | 5-3.30 PST

DIGITAL EVENT

Realize the Commercial Potential of Gene Therapies

Federico Mingozzi
CSO
Spark Therapeutics

Seng Cheng
SVP & CSO, Rare Disease Research Unit
Pfizer

Melissa Kotterman
VP, Discovery & Engineering, Co-Founder
4D Molecular Therapeutics

Peter Marks
Director, CBER
FDA

Katherine Dallow
VP, Clinical Programs & Strategy
Blue Cross Blue Shield of Massachusetts

Adam Shaywitz
CMO
BridgeBio Gene Therapy

Sam Wadsworth
CSO
Ultragenyx

Rusty Kelley
VP, Investments & Alliances
Foundation Fighting Blindness

SENIOR PARTNERS

Lonza Pharma & Biotech

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genetherapy-conference.com

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hansonwade

WELCOME TO THE 4TH ANNUAL GENE THERAPY FOR RARE DISORDERS

A Letter from our Program Director

Dear Gene Therapy Enthusiasts,

The gene therapy space has come a long way in the few short years since this conference first ran.

In the midst of such uncertainty and disruption, it's clear that the gene therapy field remains highly resilient. Remarkable progress has been made in a host of indications, with pioneering approvals on both sides of the Atlantic and skyrocketing investment in the field, demonstrating that the academic promise that has been evident for decades is finally being realized on a commercial scale.

However, it's clear that commercialization brings a whole host of unique challenges to the fore. That's why the specificity of this conference is so important. Focusing on gene therapy development in the rare disease space will lead to the kinds of in-depth discussions that will bring about tangible improvements to your gene therapy pipeline.

For these insights to be useful, they need to be delivered by people actively involved in making the decisions that matter. In a time where it can be easy to feel disconnected from our peers, now more than ever it's important to come together as a community to address the challenges we are facing. That's why for the 2021 meeting, I'm looking forward to hearing from a greater range of companies and organizations than ever before, representing the pharma, biotech, academic, payer, patient advocacy and not-for-profit organizations at the forefront of the field.

If you are or want to become a player in the gene therapy arena, this is the conference you should attend to both learn and make valuable connections that will help drive your gene therapy development forward and turn the promise of these therapeutics into a reality for patients.

I hope you can join us online in February.

Sincerely,

Alice Maynes

Program Director
Hanson Wade

Key Benefits of Attending

Implement Effective Analytical & CMC Approaches to Improve Safety, Potency & Purity

Explore technical insights into the analytical approaches supporting **Roche's** gene therapy development and understand how **bluebird bio** have aligned CMC with regulatory expectations to support an approval

Establish Robust & Scalable Manufacturing Processes & Infrastructure to Support Increasing Demand

Equip yourself for commercial manufacturing, however you utilize capacity – from making initial 'make vs buy' decisions, to CMO management and facility design, validation and maintenance, with insights from **Pfizer** and **Orchard Therapeutics**

Navigate the Complex Global Gene Therapy Regulatory Environment

Learn directly from international regulatory bodies including the **FDA** and China's **CFDA,** to understand the realities of global regulatory expectations and how regulatory oversight is adapting to technical innovations in the field

Examine Innovative Financing & Reimbursement Strategies

Understand the payer perspective on the reimbursement strategies that are available, which approaches are working, which are not and how future government action will impact pricing and value-based agreements, with insights from **Harvard Pilgrim Health** and **Blue Cross Blue Shield of Massachusetts**

Investigate Novel Vectors to Enhance Selectivity & Efficacy While Reducing Immunogenicity

Look beyond the current crop of gene therapy vectors to gain an insight into the next generation of delivery technologies with the promise to dramatically enhance the specificity of targeting, reduce costs of manufacturing and overcome immunogenicity challenges, with insights from **4D Molecular Therapeutics** and **Amarna Therapeutics**

Digital Events: An Interactive Online Experience

Gene Therapy for Rare Disorders is committed to delivering the high-quality insights and industry connections that our customers expect, in a format that is accessible from the comfort of your home or office.

We have created the virtual summit to satisfy the industry's need to share cutting-edge research, learn from your peers and engage in quality networking within a niche and highly selective audience to forge valuable collaborations.

To effectively facilitate this need to learn and connect, our custom-built virtual event platform will combine bestin- class platforms to deliver a seamless event experience. Accessing the platform is simple, you'll be provided with a unique link in the run up to the event that will take you directly to the online event space where you'll follow a few simple steps to set up your delegate profile and get started.

If you have any other questions about the platform, please visit our FAQ page

"Exciting new way of participating in scientific conference and discussion."

Vice President - New Therapeutic Targets Discovery, uniQure

Key Features & Functionalities:

Delegate Profile

Set up personalized profiles to easily identify the name, job title & company of other attendees

Stage Area

Most presentations will be delivered in the 'Stage' area, much like the main conference room onsite

Sessions Area

Smaller groups can get together in this breakout area for panel discussions and other interactive sessions

Demo Area

Visit the virtual exhibition area to explore the solutions our specialist suppliers have on offer

Chat Rooms

Connect with your peers and start conversations with individuals or all attendees in private and public chatrooms

Speed Networking

This virtual networking session will automatically connect you with other attendees to establish new industry contacts

What You Can Expect from a Digital Event:

Live Q&As with Speakers

Ask your burning questions directly to our expert speakers in real-time, just as you would at a physical conference

Audience Discussions

Join smaller, informal group chats or video calls designed to spark crucial conversations around key challenges for the industry

2+ Hours of Networking

Facilitated and informal networking breaks will allow you to connect 1-2-1 with other attendees and kick start critical discussions

Content Available Post-Event

On conclusion of the event, presentations will be made available to attendees where possible

PERSPECTIVES FROM A RANGE OF STAKEHOLDERS

Building an Industry-Leading Gene Therapy Portfolio in Rare Disease

Seng Cheng
SVP & CSO, Rare Disease Research Unit
Pfizer

Learn directly from Seng Cheng as he discusses how to build an industry-leading portfolio in rare disease, from the role of partnerships to creating the right infrastructure for the timelines you need.

Shifting Payment Paradigms: How One-Time High Cost Treatments Challenge the Traditional U.S. Payer Model

Katherine Dallow
VP, Clinical Programs & Strategy
Blue Cross Blue Shield of Massachusetts

Through a detailed case study, Katherine Dallow will demonstrate how the unique challenges gene therapies pose must be approached and how new payment frameworks must be provided.

Mitigation of Immunogenicity to Enable Re-Dosing of AAV Vectors by Co-Administration with Tolerogenic ImmTOR Nanoparticles

Kei Kishimoto
CSO
Selecta Biosciences

Revealing unpublished data, learn how Kei Kishimoto has established clinical proof-of-concept for a nanoparticle co-administration approach to inhibits the formation of anti-AAV antibodies.

KEY SESSIONS YOU CANNOT MISS

Start-Up Focus Evening

Learn how to establish yourself as a new company in the field, gain insights from innovative start-ups in the space and understand how investors view gene therapy companies through this interactive Focus Evening.

4 Tracks of In-Depth Content

Delve into the details of CMC & Analytical, Manufacturing, Clinical and Commercial gene therapy challenges through four dedicated tracks of detailed content. Engage and benchmark with peers who are encountering the same challenges as you in an intimate, interactive environment geared towards fostering long-term collaborations.

Gene Therapy 101 Full-Day Workshop

If you're new to the gene therapy field or are looking to get your staff up to speed, look no further than the comprehensive gene therapy 101 day. Establish core skills and understanding in the critical areas of gene therapy development, including the fundamentals of AAV and lentiviral vectors and the challenges gene therapy vectors impose on carrying out preclinical and clinical development.

Discussion Day: Is Gene Therapy Really a Cure? Re-Dosing: The Elephant in the Room

Gain a comprehensive insight into the immunogenicity challenges encountered in gene therapy development, before exploring the novel approaches Selecta Biosciences and the University of Florida are developing to mitigate immunogenicity and enable re-dosing.

AGENDA AT A GLANCE

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

PRE-CONFERENCE WORKSHOP DAY
Monday, February 22, 2021

- Gene Therapy 101
- A
- B
- C
- D
- Lunch
- Gene Therapy 101
- E
- F
- G
- H
- **Start-Up Focus Evening**

CONFERENCE DAY 1
Tuesday, February 23, 2021

- **The Future of Gene Therapy**
- Morning Refreshments & Networking
- **CMC & Analytical** — Analytical Assays
- **Manufacturing & Scale-Up** — Commercializing Manufacturing & Facilities
- **Clinical** — Regulatory Harmonization
- **Commercial** — Payer Perspective
- Lunch & Networking
- Characterization & Developability
- Scalable Manufacturing
- Building Trials
- Pricing Models
- Afternoon Refreshments & Networking
- **AAV & Beyond**

CONFERENCE DAY 2
Wednesday, February 24, 2021

- **Breakfast Briefing**
- **The View from the Cutting Edge**
- **CMC & Analytical** — CMC Development
- **Manufacturing & Scale-Up** — Internal vs External Manufacturing
- **Clinical** — Product Differentiation
- **Commercial** — Market Access
- Raw Materials
- Optimizing Process Development
- Patient-Centric Clinical Development
- Business Models
- **Integrating the Patient Voice**

POST-CONFERENCE DISCUSSION DAY
Thursday, February 25, 2021

- **Is Gene Therapy Really a Cure? Re-Dosing: The Elephant in the Room**
- Morning Refreshments
- **Current Re-Dosing Viability**
- Lunch
- **Immune Responses to Capsids & Transgenes**
- Afternoon Refreshments
- **Potential Re-Dosing Approaches**

YOUR EXPERT SPEAKERS

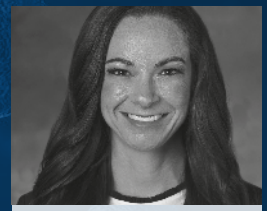
Luca Alberici
CBO
MolMed

Leone Atkinson
Executive Medical Director, Rare Disease & Pediatrics Team
Covance

Gilles Atlan
VP
NF2 BioSolutions

Robert Baffi
President, Global Manufacturing & Technical Operations
BioMarin

Grant Blouse
SVP, Translational Research
Catalyst Biosciences

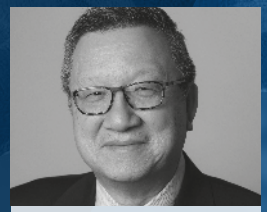


Allison Bradbury
Principal Investigator
Nationwide Children's Hospital

Lucas de Breed
Managing Director
AUGC

Scott Burger
Principal
Advanced Cell & Gene Therapy

Mindy Cameron
Advocacy Director
Little Hercules Foundation



Seng Cheng
SVP & CSO, Rare Disease Research Unit
Pfizer

Vivian Choi
Head of Gene Therapy Research
Takeda

Daniel Chung
Global Medical Lead, Ophthalmology
Spark Therapeutics

Manuela Corti
Assistant Professor
University of Florida

Nick Crabb
Programme Director, Scientific Affairs
NICE

Katherine Dallow
VP, Clinical Programs & Strategy
Blue Cross Blue Shield of Massachusetts

Doug Danison
VP, Head of Access, Value & Evidence Strategy (AVES)
bluebird bio

Olivier Danos
CSO
REGENXBIO

Kendall Davis
Manager, Patient Advocacy & Engagement Strategy, Center for Rare Diseases
PRA Health Sciences

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YOUR EXPERT SPEAKERS

Jill Dolgin
Head of Patient Advocacy
AGTC

Anne Dupraz-Poiseau
Chief Regulatory Officer
Orchard Therapeutics

David Favre
Vice President, Translational Medicine
Asklepios BioPharmaceutical

Alison Finger
CCO
bluebird bio

Jayne Gershkowitz
Chief Patient Advocate
Amicus Therapeutics

Clive Glover
Director, Cell & Gene Therapy
Pall Biotech

Steven Goodman
Head, Drug Product Manufacturing
bluebird bio

Géraldine Guérin-Peyrou
Director of Marketing & Communication
Polyplus Transfection

Peter de Haan
Chief Scientific Officer & Co-Founder
Amarna Therapeutics

Nandini Hadker
Trinity

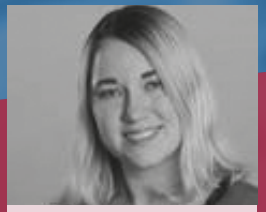
Markus Haindl
Lead Gene Therapy Technical Development
Roche

Marianne Hamilton Lopez
Director, Research
Duke-Margolis Center for Health Policy

Jessica Hilmoe
Technical Sales Manager, Manufacturing & Operations
MilliporeSigma

Nina Hunter
VP, Regulatory Affairs & Science Policy
REGENXBIO

Vibha Jawa
Director & Lead
Merck



Angela Johnson
Senior Director, Regulatory Affairs
Sigilon Therapeutics

Tony Khoury
VP, Technical Services
Project Farma

Anna Kaltenboeck
Program Director, Center for Health Policy & Outcomes, Drug Pricing Lab
Memorial Sloan Kettering Cancer Center

Rusty Kelley
VP, Investments & Alliances
Foundation Fighting Blindness

Sven Kili
Principal
Sven Kili Consulting

Kathleen Kirby
SVP, Development Operations
BridgeBio Gene Therapy

YOUR EXPERT SPEAKERS

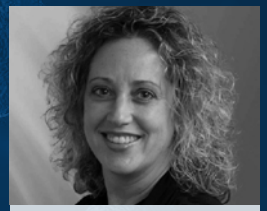
Kei Kishimoto
CSO
Selecta Biosciences

Dave Knop
Executive Director, Process Development
AGTC

Melissa Kotterman
VP, Discovery & Engineering, Co-Founder
4D Molecular Therapeutics

Janet Lynch Lambert
CEO
Alliance for Regenerative Medicine (ARM)

Larissa Lapteva
Associate Director, OTAT, CBER
FDA

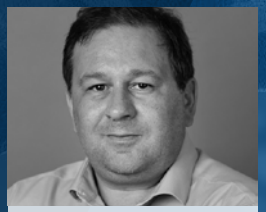


Mindy Leland
Gene Therapy Quality Operations Team Lead
Pfizer

Julie Lin
Global Project Head of Early Clinical & Business Development, Rare Diseases
Sanofi

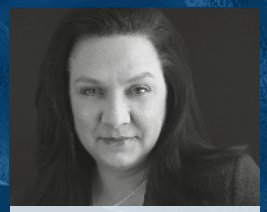
Xiaohui Lu
Director, Analytical Development
Ultragenyx

Peter Marks
Director, CBER
FDA



David Markusic
Assistant Research Professor of Paediatrics
IUPUI

Chris Mason
CSO
AVROBIO



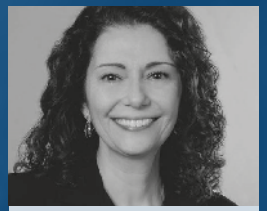
Kelly Maynard
President & Founder Little Hercules
Foundation

Jesse McCool
CTO
Cytovance Biologic

Emily McGinnis
Chief Patient Officer & Head of Government Affairs
Taysha Gene Therapies

Emily Milligan
Executive Director
Barth Syndrome Foundation

Federico Mingozzi
CSO
Spark Therapeutics

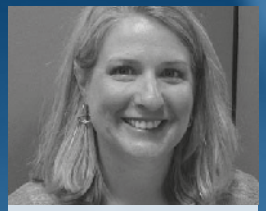


Christina Ohnsman
Senior Clinical Development Lead
REGENXBIO

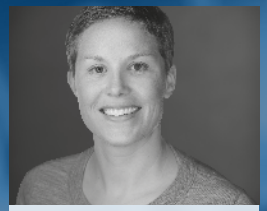
Thomas Page
VP, Engineering & Asset Development
Fujifilm Diosynth

YOUR EXPERT SPEAKERS

Palani Palaniappan
Chief Technology Officer
Aruvant



Shelly Parra
Director, Global Field Applications
Repligen



Krista Perry
Principal, Head of Launch Excellence
Trinity

Markus Peters
Consultant
Independent

Leslie Powell
Director, Policy & Advocacy
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Kim Raineri
Chief Manufacturing & Technology Officer
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Amy Raymond
Director of Therapeutic Expertise, Center for Rare Diseases
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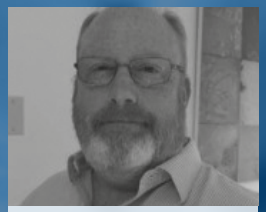
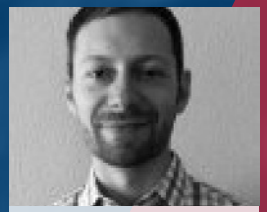


Jim Richardson
Senior Scientific Liaison
US Pharmacopeia



Patrick Rivers
Director of Research
Aquilo Capital Management

Christian Rubio
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BridgeBio Gene Therapy

Michael Sherman
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Devyn Smith
COO & Head of Strategy
Sigilon Therapeutics

William Smith
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Pioneer Institute

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Ales Strancar
Managing Director
BIA Separations

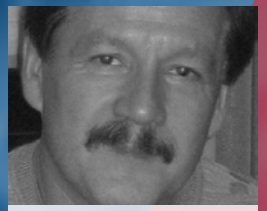
Paulina Szymanska
Senior Account Manager
Beacon Targeted Therapies

YOUR EXPERT SPEAKERS

Magali Taiel
Chief Medical Officer
GenSight Biologics

Mark Trusheim
Strategic Director, NEWDIGS & Visiting Scientist
MIT

Mauricio Umana
Associate Director, Global Regulatory CMC Lead
Biogen



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Ultragenyx Pharmaceuticals

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Sr Advisor, Cell & Gene Therapy Manufacturing
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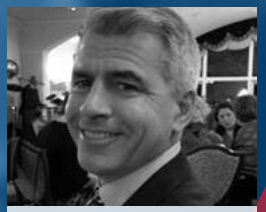

Lance Weed
Consulting
Lance Weed Consulting

Alex Xu
Chief Scientist
CFDA/CDE

Nora Yang
Chief Scientific Officer
Stratify Therapeutics

Frank Zhang
VP, Pricing, Market Access, Value & Policy
uniQure

Yuan Zhao
Leader, Gene Therapy Section
NIBSC, MHRA

Voyager THERAPEUTICS

Speaker to be confirmed
Voyager Therapeutics

CareMetx

Speaker to be confirmed
CareMetx

ThermoFisher SCIENTIFIC

Kye Ehart
Head of Process Development & Viral Vector Services
Thermo Fisher Scientific

INVITAE

Gautam Mehta
Head of Biopharma Business Development
Invitae

The Jackson Laboratory

Speaker to be confirmed
The Jackson Laboratory

BIO-RAD

Speaker to be confirmed
Bio-Rad

PRE-CONFERENCE WORKSHOPS - MONDAY, FEBRUARY 22 AM

All times are in EST

WORKSHOP A - 9:00AM-12:00PM

Discussing the Value of Standards in the Gene Therapy Field & Sharing the World Health Organisation (WHO) International Gene Therapy Standards in Development

Establishing relevant and applicable standards to apply to gene therapy development is essential in maintaining safe and effective therapeutics. This also provides clarity and consistency for companies developing these therapeutics, especially as gene therapies progress into the late stages of development. Setting standards is especially challenging in the gene therapy space, where changes to manufacturing processes are frequent and the development is in orphan indications, limiting the possibilities for comparison with other approaches.

Attend this workshop to learn about:

- The development of international World Health Organization standards for lentiviral gene therapies
- The current standards that exist for AAV therapeutics and the kinds of additional standards that are required
- The importance of safety and potency standards
- Data generated from the development of gene therapy standards, as well as research into the development of new assays and control testing of gene therapies

Workshop Leader:

Yuan Zhao
Leader, Gene Therapy Section,
NIBSC, MHRA

WORKSHOP B - 9:00AM-12:00PM

Building Internal Gene Therapy Manufacturing Capacity

Robust, scalable and safe gene therapy manufacturing relies both on appropriate processes and facilities. With an increasing number of gene therapies progressing into late-stage development, the requirement for manufacturing capacity has brought facility design to the top of the agenda.

Attend this workshop to learn about:

- Optimizing scalable manufacturing practices to scale up to commercial manufacturing
- Understanding the regulatory requirements for gene therapy facilities and how internal builds and external contract facilities can ensure they meet these standards
- Designing flexible manufacturing facilities to adapt to future demands
- Discussing case studies from previous vector manufacturing facilities: what can be learnt to positively impact the future of the field?

Workshop Leader:

Lance Weed
Consulting
Lance Weed Consulting

WORKSHOP C - 9:00AM-12:00PM

Meaningful Clinical Endpoints in Rare Disorder Trials

Establishing clear, meaningful and rigorous clinical endpoints is essential in any clinical trial. The variability across patient populations, small trial numbers, and limited data on natural histories of rare diseases makes identifying and validating endpoints in rare disorder trials difficult. With more gene therapies in the clinic than ever before, it is important to identify clinical endpoints in a robust and worthwhile way.

Attend this workshop to learn about:

- Tailoring clinical endpoints where classical endpoints may not be suitable
- Acknowledging and assigning surrogate endpoints
- Identifying and validating meaningful endpoints for the patient
- Maximising outcome endpoints with the fewest number of patients
- Setting a precedent for a primary endpoint

Workshop Leaders:

Larissa Lapteva
Associate Director, OTAT, CBER
FDA

Christina Ohnsman
Senior Clinical Development Lead
REGENXBIO

WORKSHOP D - 9:00AM-12:00PM

Evaluating the Realities of Value-Based Payment Models

The ever-expanding pipeline of innovative yet costly therapeutics, like gene therapies, is causing existing payment approaches to be reconsidered so they can support better outcomes for patients as well as better value across the system. Value-based payments can link products to outcomes such as clinical measurements and patient-reported outcomes. This workshop will focus on the real-world applications of value-based payment models and how these approaches can best be implemented in the near future.

Attend this workshop to learn about:

- The implementation of a framework to better define when outcomes-based approaches are most appropriate
- Understanding how barriers to the adoption of innovative new therapeutics can be reduced
- Investigating the legal and regulatory challenges specific to the gene therapy space
- Gaining insights into the practical realities of different payment models: what do they mean to the patients?
- What would an outcomes-based model look like within Medicaid at the state level?
- How can mechanisms for outcomes-based models at Medicare be developed within current regulatory capabilities?

Workshop Leader:

Marianne Hamilton Lopez
Director, Research
Duke- Margolis Center for Health Policy

PRE-CONFERENCE WORKSHOPS - MONDAY, FEBRUARY 22 AM

All times are in EST

WORKSHOP E - 1:00PM-4:00PM

Unifying Process & Analytical Development to Establish Robust, Scalable Gene Therapy Manufacturing Processes

Establishing scalable manufacturing processes from early in development has a lot of advantages, but is complex, requiring the integration of analytical, process development and regulatory expertise. It is also a balancing act, requiring thorough analysis of development and clinical timelines, as well as the constantly evolving regulatory landscape, to ensure that processes are established in a timely and effective manner. This workshop will examine this topic, sharing insights into the establishment of scalable gene therapy processes that will pre-emptively overcome challenges in future scale-up to commercialization.

Attend this workshop to learn about:

- How analytical development can function in parallel with process development to shorten timelines and decrease costs
- Approaches to stay ahead of regulatory guidances in an ever-evolving regulatory environment
- Examining case studies detailing process development and analytical challenges encountered in the development of AAV-based gene therapy products

Workshop Leader:

Dave Knop
Executive Director, Process Development, **AGTC**

WORKSHOP F - 1:00PM-4:00PM

Enhancing the Outsourcing Process: How to Select & Manage Contract Manufacturing Services & the Broader Contract Services Ecosystem Effectively

The days of small-scale academic work in the gene therapy field are morphing into the early stages of the commercial era. For this reason, facilitating successful outsourcing relationships with contract manufacturers, testing labs and logistics companies becomes more essential than ever before, in order to support large-scale commercial manufacturing on a global scale.

Attend this workshop to learn about:

- What constitutes a good CMO? Investigating the criteria you should be using to adequately assess potential partners to outsource projects to in the gene therapy space
- The broader contract infrastructure – understand how to improve interactions with CMOs, CROs, contract testing firms and contract shipping and transportation companies
- Key factors impacting the decision to develop in-house capabilities or outsource services to third parties
- Highlighting the importance of quality agreements from both parties in establishing successful collaborations

Workshop Leader:

Scott Burger
Principal
Advanced Cell & Gene Therapy

WORKSHOP G - 1:00PM-4:00PM

Utilizing Pre-Clinical Data to Overcome the Translational Gap to the Clinic

Pre-clinical work remains the foundation of creating a successful gene therapy. Without robust, accurate, high quality data from pre-clinical studies, it is difficult to progress to the clinic with a likely candidate for a successful therapy. A lot can be learned from pre-clinical data, and maximising the output from those studies is key to paving the way to the clinic.

Attend this workshop to learn about:

- Establishing efficacy in humans based on animal models by choosing the right preclinical readouts
- Getting the dose right. How best to scale dose from animal models to human patients
- Modeling the route of administration and biodistribution challenges in preclinical models
- Facing immunogenicity concerns, use of immunosuppression, and the possibility of redosing in animal models
- Safety and toxicity testing in animal models. What's the right pre-clinical safety species?
- Instituting good laboratory practices: The importance of quality control in reporting animal data

Workshop Leader:

Allison Bradbury
Principal Investigator
Nationwide Children's Hospital

WORKSHOP H - 1:00PM-4:00PM

Post-Approval Commercialization: Understanding the Realities of the Post-Approval Landscape.

2018 was the first year that there were more orphan drugs approved than non-orphan drugs by the FDA. A revolution is happening in the biopharmaceutical business model: it's shifted from blockbuster drugs to drugs for smaller and smaller patient populations. This causes many constantly evolving challenges to the policy and payer communities, many of which are very specific to certain indications, locations and patient circumstances. With the situation only set to increase in complexity given the ever-expanding pipeline of gene therapies, discussions around the emerging issues facing the space are essential.

Attend this workshop to learn about:

- The changes that need to be made to adjust to the new realities of biopharmaceutical pipelines
- Establishing benchmarks for value and pricing decisions in a variety of therapeutic areas
- Discussing how appropriate the QALY approach is to assess the cost-effectiveness of orphan drugs
- What are the key aspects that need to be considered when pricing gene therapies?
- Investigating approaches to include a wide variety of societal factors from patients, physicians and caregivers in determining the value of gene therapy products.

Workshop Leader:

William Smith
Visiting Fellow On Life Sciences
Pioneer Institute

GENE THERAPY 101 DAY

MONDAY, FEBRUARY 22

10.00AM - 3.00PM EST | 7.00AM - 12.00PM PST

"Very well organized and informative virtual conference!"

Senior Director - Regulatory Affairs, Adverum

Your Comprehensive Introduction to the Gene Therapy Space

Are you new to the gene therapy field?

Looking to get up to speed on the nuances of developing gene therapies in the rare disease space?

This full-day workshop will offer an intense learning environment to establish core skills and understanding in the critical areas of gene therapy development. Designed for new entrants to the gene therapy field, it will deliver critical knowledge on a number of unique challenges encountered when working with gene therapies.

Covering essential elements, this workshop will enable you to:

- Understand the fundamentals of AAV and lentiviral vectors
- Transition effectively from academia into industry
- Gain clarity into the ever-evolving regulatory landscape
- Investigate the challenges gene therapy vectors impose on carrying out preclinical and clinical gene therapy work
- Learn the transferrable insights that can be leveraged in the gene therapy space from working with other therapeutics
- Uncover valuable information from the past: using the history and acquired learnings from the field to inform future progress
- Guarantee safety at every stage of development
- Integrate the patient voice into gene therapy development and accessing a patient advocacy toolkit

Chaired by:

Sven Kili
Principal
Sven Kili Consulting

Speakers Confirmed to Contribute So Far Include:

Karen Walker
Sr Advisor, Cell and Gene Therapy Manufacturing
Genentech

Grant Blouse
SVP, Translational Research
Catalyst Biosciences

Nina Hunter
VP, Regulatory Affairs & Science Policy
REGENXBIO

Markus Peters
Consultant
Independent

Christian Rubio
VP, Strategic Advancement
Global Genes

Devyn Smith
COO & Head of Strategy
Sigilon Therapeutics

Dan Wang
Assistant Professor
University of Massachusetts Medical School

Kim Raineri
Chief Manufacturing & Technology Officer
AVROBIO

genetherapy-conference.com

T +1 617 455 4188 E info@hansonwade.com Gene Therapy for Rare Disorders

hansonwade

PRE-CONFERENCE START-UP FOCUS EVENING

MONDAY, FEBRUARY 22

4.45PM - 6.30PM EST | 1.45PM - 3.30PM PST

ADDITIONAL NETWORKING OPPORTUNITIES IN THE MAIN AGENDA

POSTER SESSION

Virtually share your work with the community and gain feedback on your latest gene therapy research. Open to all attendees, the scientific poster session is an invaluable opportunity for you to review pioneering gene therapy work being carried out by your peers, as well as presenting your own research. All posters are subject to approval by the organizers.

SPEED NETWORKING

This unique virtual networking session will enable you to engage with a large proportion of your fellow delegates early in the conference. Rapidly establish the connections you want to pursue and relationships you want to forge over the rest of the conference and beyond.

What Does it Take to Succeed as an Early-Stage Gene Therapy Company?

What challenges have key investors and start-up companies in this field faced and how have they overcome them? What are investors looking for? These are just some of the questions that this dedicated focus evening will address, enabling you to understand how young companies are establishing themselves in this rapidly-evolving field and how funding and investment can be accessed to accelerate innovation.

Gain insights and perspectives from investors who have been pivotal in establishing some of the most innovative gene therapy companies and leave with actionable insights to kick-start progress at your company, however established you are in the space.

Patrick Rivers
Director of Research
Aquilo Capital Management

Rusty Kelley
VP, Investments & Alliances
Foundation Fighting Blindness

Lucas de Breed
Managing Director
AUGC

AGENDA

4:45pm Chair's Opening Remarks

5:00pm Building Your Value Story from Day -1
Lucas de Breed, *Managing Director,* **AUGC**

5:15pm Manufacturing Considerations for Gene Therapy Investment
Patrick Rivers, *Director of Research,* **Aquilo Capital Management**

5:30pm Funding & Investing in Early-Stage Ophthalmic Gene Therapy Start-Up Companies
Rusty Kelley, *VP, Investments & Alliances,* **Foundation Fighting Blindness**

5:45pm Panel Discussion
Collectively discuss the gene therapy field and share experiences in overcoming key early stage challenges in a variety of indications. This is your opportunity to engage with these experts and have your questions answered in an engaging, intimate session.
Philip Reilly, *Venture Partner,* **Third Rock Ventures**
Rusty Kelley, *VP, Investments & Alliances,* **Foundation Fighting Blindness**
Patrick Rivers, *Director of Research,* **Aquilo Capital Management**
Lucas de Breed, *Managing Director,* **AUGC**

6:30pm Chair's Closing Remarks

CONFERENCE DAY ONE **TUESDAY, FEBRUARY 23**

All times are in EST

7:50am **Chair's Opening Remarks**

Chris Mason
CSO
AVROBIO

Pioneering the Next Generation of Gene Therapies

8.00am **Keynote: FDA's Efforts to Enhance Gene Therapy Regulatory Interactions**

- Sharing FDA considerations regarding product manufacturing, including process validation
- Understanding how regulatory oversight will need to adapt to keep pace with technical innovations in gene therapy manufacturing and development
- Introducing the CATT meeting – an additional early opportunity to engage with regulators around key issues encountered in the broader development of gene therapies
- Distinguishing how CATT meetings are different from INTERACT meetings

Peter Marks
Director, CBER
FDA

8.30am **Building an Industry-Leading Therapy Portfolio in Rare Disease**

- Working strategy and future commitment to gene therapy of rare diseases in Pfizer
- Role of external partnerships and collaborations for a sustainable gene therapy pipeline
- Infrastructure and supporting elements to support timely and broad access to emerging gene medicines

Seng Cheng
SVP & CSO, Rare Disease Research Unit
Pfizer

9.00am **Industry Leaders Panel: Delivering on the Promise of Gene Therapies**

Gene therapies continue to redefine the treatment of rare diseases, but these conditions present many distinct development challenges. This panel discussion will bring together experts from the leading pharma and biotech companies pioneering this field to analyse how we can overcome these hurdles to realize the commercial potential of gene therapies

- Navigating uncharted territory: sharing the decisions industry pioneers have made to pioneer progress without a defined pathway
- Changing the mind-set: understanding how the unique characteristics of gene therapies are in the process of changing the healthcare paradigm and how companies have to adapt to this new landscape
- Gearing up for commercialization: sharing how the leading companies are preparing for a new wave of gene therapies

Sam Wadsworth
CSO
Ultragenyx Pharmaceuticals

Peter Marks
Director, CBER
FDA

Robert Baffi
President, Global Manufacturing & Technical Operations
BioMarin

Seng Cheng
SVP & CSO, Rare Disease Research Unit
Pfizer

Alison Finger
CCO
bluebird bio

9.30am **Speaking position reserved for Lonza**

Lonza
Pharma & Biotech

10:00am Morning Refreshments & Speed Networking

Grab a quick cup of tea or coffee from the comfort of your own kitchen and jump straight into your opportunity to connect with new contacts from active companies in the field and exchange digital business cards. Network and form lasting connections through this exclusive virtual speed networking!

CONFERENCE DAY ONE **TUESDAY, FEBRUARY 23**

All times are in EST

CMC & ANALYTICAL
Establishing Analytical Assays to Assess Critical Quality Attributes

11.00am Considerations Towards Technical Development of Viral Vector-Based Gene Therapies

- Technical Development for viral vector-based gene therapies: which approaches and elements could be and which ones shouldn't be transferred from MABs
- Development of a stable cell lines toolkit to put industrialization of viral vector-based gene therapies to another level
- Tackling the Bioassay challenge: applying a phase appropriate approach, surrogate strategies, and the matrix concept

Markus Haindl, *Lead Gene Therapy Technical Development,* **Roche**

11.30am Next-Generation Transfection Reagent for Large Scale AAV Manufacturing

- Transient transfection of suspension cells is the most commonly used method for AAV manufacturing
- However, this method shows some limitations when upscaling the process
- To address this concern, we developed a novel transfection reagent showing increased AAV titers and improved transfection protocol for large scale bioreactors

Géraldine Guérin-Peyrou, *Director of Marketing & Communication,* **Polyplus Transfection**

MANUFACTURING SCALE-UP
Making Informed Strategic Manufacturing Decisions and Designing Effective Facilities

11.00am Standardizing Gene Therapy Manufacturing Facilities

- Establishing standards and implementing these in internal and external facilities
- Prioritizing standards and communicating this effectively to partners
- Understanding the knock-on effects of differing quality standard on overall program development

Speaker to be confirmed

11.30am Quality & Regulatory Requirements from Clinical to Commercial Manufacturing

- Keeping pace with the evolving regulations and interacting effectively with regulatory colleagues and agencies
- Understanding the regulatory requirements for a gene therapy multi-product manufacturing facility
- Managing the unique quality challenges encountered in the manufacturing of gene therapy products and scale up

Mindy Leland, *Gene Therapy Quality Operations Team Lead,* **Pfizer**

CLINICAL
Exploring the Continually Evolving Regulatory Landscape

11.00am A Strategic Playbook for Key Rare Disease Gene Therapy Regulatory Milestones

- Examine evolution of type and standard of data required by regulatory authorities and key precedent products
- Highlighting the success criteria at key milestones throughout development
- Discussing strategic trial designs for rare and ultra-rare disorders in the context of outcomes and endpoints

Angela Johnson, *Senior Director, Regulatory Affairs,* **Sigilon Therapeutics**

11.30am Harmonizing a Global Program for Gene Therapies

- Gaining insights into creating a global program
- Understanding regulatory requirements for gene therapy development in Europe and the USA
- Leveraging lessons learned from previous approvals in both geographies
- Identifying opportunities/ hurdles for accelerated global development

Anne Dupraz-Poiseau, *Chief Regulatory Officer,* **Orchard Therapeutics**

COMMERCIAL
Understanding the Payer Perspective

11.00am Shifting Payment Paradigms: How One-Time High Cost Treatments Challenge the Traditional U.S. Payer Model

- Gaining insights into the unique challenges gene therapies pose to the classical payer model
- Providing a framework for how payers need to consider the balance between access and affordability in the context of innovative but expensive therapeutics
- Understanding the traditional stop loss financing model for high cost cases and how that model is not sustainable for gene therapies
- Case Study: Self-funded account and cumulative gene therapy cost impacts on stop loss

Katherine Dallow, *VP, Clinical Programs & Strategy,* **Blue Cross Blue Shield of Massachusetts**

11.30am Payer Perspective on How Innovative Financing for Gene Therapies is Evolving

- Examining financing strategies that are working, those that are not and how payers are course-correcting
- Understanding the nuances of pricing strategies and exploring emerging models
- How might government action help or hinder pricing and value-based agreements?
- Case study: Using Zolgensma as a demonstration of the consequences of uncertainty around the FDA label

Michael Sherman, *CMO & SVP,* **Harvard Pilgrim Healthcare Institute**

CONFERENCE DAY ONE **TUESDAY, FEBRUARY 23**

All times are in EST

12.00pm **Analytical Method Development & Product Characterizations to Support**
the Late Stage Development of Gene Therapy Products

- New analytical methods to ensure product quality and process consistency
- Gaining valuable biophysical characterization from technologies such as AUC and SEC
- Advanced product characterization to ensure comparability

Xiaohui Lu, *Director, Analytical Development,* **Ultragenyx**

12.00pm **Industrializing Gene Therapies: The Role of Manufacturing Technologies**

- Successful commercialization of gene therapies is dependent on robust, consistent manufacturing methods that are appropriate for the scale required for the targeted indication
- Current manufacturing is often dependent on technologies that cannot be scaled in a series of disconnected unit operations reducing consistency and robustness
- This talk will demonstrate the creation of a full end-to-end, scalable platform solution for viral vector manufacturing
- This solution can be deployed rapidly allowing therapies to get to market rapidly

Clive Glover, *Director, Cell & Gene Therapy,* **Pall Biotech**

12.00pm **Embedding Advocacy and Engagement in Your Clinical Development Program**

- Patient organization engagement roadmap
- Critical success factors for integrating patient perspective into the development plan
- Real-world insights into applications

Kendall Davis, *Manager, Patient Advocacy & Engagement Strategy,* **Center for Rare Diseases, PRA Health Sciences**
Amy Raymond, *Director of Therapeutic Expertise, Center for Rare Diseases,* **PRA Health Sciences**

12.00pm **The Evaluation of Cell and Gene Therapies**

- To outline the challenges of applying health technology assessment to cell and gene therapies
- To consider the need for managed access arrangements
- To provide case histories from NICE's experience

Nick Crabb, *Programme Director, Scientific Affairs,* **NICE**

12:30pm **Speaking Position Reserved for Bio-Rad**

BIO-RAD

12.30pm **Make vs. Buy Considerations in the Gene and Cell Therapy Space**

- Capital vs operational expenditures associated with the existing strategies
- Implications to intellectual property and quality control
- Remaining solution-driven in the face of tight timelines

Jesse McCool, *CTO,* **Cytovance Biologic**
Tony Khoury, *VP, Technical Services,* **Project Farma**

12.30pm **PANEL DISCUSSION: Investigating the Current Global Regulatory Landscape in Light of Recent Approvals**

- Learning from previous approvals to shape standards for future approvals
- Emphasizing safe and responsible development of gene therapies
- Exploring impacts on regulation of increased numbers of gene therapies looking for approval

Anne Dupraz-Poiseau, *Chief Regulatory Officer,* **Orchard Therapeutics**
Peter Marks, *CBER,* **FDA**
Alex Xu, *Chief Scientist,* **CFDA/CDE**
Aron Stein, *VP Global Regulatory Affairs,* **Sangamo Therapeutics**

12:30pm **Speaking Position Reserved for CareMetx**

CareMetx

1:00pm Networking Lunch

CONFERENCE DAY ONE **TUESDAY, FEBRUARY 23**

All times are in EST

CMC & ANALYTICAL
Enhancing Analytical Characterization & Developability Assessments

2.00pm **Keeping CMC Activities Off the Critical Path for Gene Therapy Development**
- Accelerating AAV-based gene therapy requires a strategic development plan
- Advantages of early investment in CMC activities
- Improving AAV manufacturability
- Balancing speed-to-market and product quality

Rajiv Gangurde, *Senior Director, CMC Strategy & Execution,* **Voyager Therapeutics**

2.30pm **Rapid CMC Development & Pre-Commercial Considerations for rAAV Gene Therapy Products for Rare Diseases**
- Establishing a fast-to- clinic CMC development strategy
- Increasing volumetric productivity and improving yields across chromatography and filtration steps
- Demonstrating scalability to 250L within 3 months post-receipt of new expression cassette
- Developing analytical characterization methods to enable product quality assessment to maintain comparability and enhance product understanding

James Warren, *VP, Pharmaceutical Development,* **Ultragenyx**

MANUFACTURING SCALE-UP
Establishing Scalable Manufacturing Processes

2:00pm **Speaking Position Reserved for Thermo Fisher Scientific**

ThermoFisher SCIENTIFIC

2.30pm **PANEL DISCUSSION: Selecting & Implementing the Most Appropriate Methodologies to Ensure Robust, Cost-Effective & Scalable Manufacturing**

It's evident that to scale-up any manufacturing within gene therapy, two key considerations are cost-effectiveness of the process, as well as how robust the process is itself. This panel will discuss how to identify the most appropriate methodologies for certain indications, and talk about how to go on to implement these effectively to save both time and money in the manufacturing process.

CLINICAL
Building Clinical Trials to Maximize Meaningful Output

2.00pm **Extracting Meaningful Data from Clinical Trials to Present to Regulatory Authorities**
- Developing a data extraction strategy and tool for data collection, taking regulators and patients into account
- Enabling successful medical record collection to support robust collection of data and managing extraction during a pandemic
- Setting up the team to analyze the extracted data and to make sense of it

Kathleen Kirby, *Senior Vice President, Development Operations,* **BridgeBio Gene Therapy**

2.30pm **Recognizing the Best Clinical Candidates for a Gene Therapy**
- Identifying the best clinical candidate for specific indications
- Establishing approaches that could robustly select vectors on a case-by-case basis
- Investing in vectors with the best long-term outcomes

Vivian Choi, *Head of Global Gene Therapy Research,* **Takeda**

COMMERCIAL
Evaluating Gene Therapy Pricing & Reimbursement Models

2.00pm **Precision Financing Framework and Pilots: Latest Finding from the MIT FoCUS Consortium**
- US federal policy limitations on reimbursement innovation
- US precision financing in action
- Administrative and execution challenges of reimbursement – discussing patient mobility and tracking issues
- Is Europe leading the US in financing orphan therapeutics?

Mark Trusheim, *Strategic Director, NEWDIGS & Visiting Scientist,* **MIT**

2.30pm **Pricing & Reimbursement Strategies for a Hemophilia B Gene Therapy Launch**
- How do we show value of the product to different stakeholders?
- Exploring potential pricing strategies and how to implement this in practice
- Understanding different payment model dynamics and how this links to patient access

Frank Zhang, *VP, Pricing, Market Access, Value & Policy,* **uniQure**

3.00pm **PANEL DISCUSSION: Understanding Manufacturing and CMC Parameters that Require Standardization**

- Reducing variability of assays – what approaches are involved
- Updating historically variable assays such as the TCID50 infectivity assay – what alternatives are there?
- Investigating automation as an approach to reduce variability

Thomas Page, *VP, Engineering & Asset Development,* **Fujifilm Diosynth**
Karen Walker, *Sr Advisor, Cell and Gene Therapy Manufacturing,* **Genentech**
Mauricio Umana, *Associate Director, Global Regulatory CMC Lead,* **Biogen**

- Insights into process development efforts to achieve greater transfection efficiency
- Upstream optimizations involved in transitioning between manufacturing processes
- Cutting through outdated information to give a realistic impression of the cost-effectiveness of manufacturing platforms

Luca Alberici, *CBO,* **MolMed**
Additional speakers to be confirmed

3.00pm **PANEL DISCUSSION: Evaluating Challenges Encountered in Small Patient Number Trials**

- Discussing how to choose the most suitable comparator
- Emphasizing the importance of understanding the natural history of the disease
- Examining likelihood of randomised placebo-controlled studies in rare disorder trials

Daniel Chung, *Global Medical Lead, Ophthalmology,* **Spark Therapeutics**
Julie Lin, *Global Project Head of Early Clinical & Business Development, Rare Diseases,* **Sanofi**
Adam Shaywitz, *CMO,* **BridgeBio Gene Therapy**
Speaker to be Confirmed, **Invitae**

3.00pm **PANEL DISCUSSION: Collecting & Utilizing Data to Support Gene Therapy Market Access**

- Understanding approaches ensure appropriate data is collected at an appropriate time to support market access decision making Investigating approaches to measure the performance of data gathered
- Reality check: discussing how to demonstrate efficacy in real life and incorporating the patient perspective into market access decision making

Mark Trusheim, *Strategic Director, NEWDIGS & Visiting Scientist,* **MIT**
Additional speakers to be confirmed

3.30pm **Virtual Scientific Poster Session**

The Poster Session is an informal part of the conference agenda, allowing you to connect with your peers using our online platform and continue to forge new and existing relationships. During this session over 25 scientific posters will be presented, showcasing the latest breakthroughs in the field and giving you the opportunity to gain an insight into the future of the field.

Investigating Novel Vector Technologies With the Promise of Enhancing Gene Delivery

4.30pm **3rd Generation AAV Manufacturing Platform & In-Process Controls**

- A non-affinity AAV manufacturing process to manage formation of complexes and result in extra low hcDNA contaminants will be presented
- New column to better separate empty and full AAV particles will be introduced
- Rapid and sensitive analytics for in-process control of AAV manufacturing using HPLC and different in-line detectors will be shown
- New ultra-sensitive DNA assay showing major AAV-associated DNA contamination will be presented

Ales Strancar
Managing Director
BIA Separations

CONFERENCE DAY ONE **TUESDAY, FEBRUARY 23**

All times are in EST

5.00pm **Improving AAV Capsids Using Directed Evolution**

- Current AAV serotypes have relatively modest tissue selectivity. Not all tissue types can be effectively transduced
- Directed Evolution can dramatically enhance AAV preference and lead to effective transduction of otherwise unavailable tissue types
- Promoter engineering can further enhance specificity
- These "Next Generation" vectors have the potential to broaden the effective and safe use of gene therapy

Melissa Kotterman
Vice President of Discovery & Engineering, Co-Founder
4D Molecular Therapeutics

5.30pm **SV40-Based Gene Delivery Vectors as an Alternative to AAV Vectors for In Vivo Gene Therapy**

- Replication-defective Simian Virus 40 (SV40)-based gene delivery vectors are safe
- SV40 vectors are non-immunogenic and unique in their capacity to induce immune tolerance to the transgene products in humans
- SV40 vectors are an attractive alternative to AAV vectors for clinical in vivo gene therapy

Peter de Haan
Chief Scientific Officer & Co-Founder
Amarna Therapeutics

6.00pm **Chair's Closing Remarks**

Chris Mason
CSO
AVROBIO

"Good mixture of excellent talks and discussions! Almost like being on a face to face conference!"

Chief Research & Development Officer, CombiGene

"I think what was here was excellent. So, don't change it. Especially the software."

Executive Director, Sarepta Therapeutics

"I have to say that I was initially sceptical of an online meeting, but it was so well organized that it almost felt like you were there in person and the sessions moved flawlessly."

Head of Search and Evaluation, Cerevel Therapeutics

The View from The Cutting Edge: Gaining the Perspective of the Industry Leaders

Breakfast Briefing Hosted by Pall Biotech

PALL Biotech

8.20am **Chair's Opening Remarks**

Chris Mason
CSO
AVROBIO

8.30am **Lumevoq Gene Therapy in Leber Hereditary Optic Neuropathy (LHON)**

- LHON is a maternally inherited mitochondrial disease. It is a rare disease, with an incidence of 1500 new patients per year in Europe and in the US. The 11778 mutation on the ND4 mitochondrial gene is the mutation causing the most severe clinical form of LHON. Lumevoq specifically targets this mutation
- Lumevoq is a recombinant adenovirus vector type 2, containing the wild type ND4 DNA. Lumevoq is injected via Intravitreal Injection
- GenSight Biologics have filed for EMA registration in September 2020, and then be moving to BLA for US registration in 2021

Magali Taiel
Chief Medical Officer
GenSight Biologics

9.00am **What About the Long-Run? Questions we Should be Asking About Cell & Gene Therapy Reimbursement**

- The first cell and gene therapies to launch in the US have set a high bar for pricing, creating challenges for existing reimbursement
- practices
- Novel financing approaches and outcomes-based contracts have been proposed as solutions to prevent the high price points of these
- therapies from acting as barriers to access
- However, these approaches do not address important uncertainties about the performance of new treatments, the potential long-term
- consequences of their wide-scale adoption, and incentives for future innovation

Anna Kaltenboeck
Program Director, Center for Health Policy and Outcomes, Drug Pricing Lab
Memorial Sloan Kettering Cancer Center

9.30am **Tactical Implementation of Patient- Centricity in Gene Therapy Development Programs**

Leone Atkinson
Executive Medical Director, Rare Disease & Pediatrics Team
Covance

10.00am **Setting Out the Gene Therapy Landscape: Where Are We Now?**

- Establishing the context and understanding how far the field has come in just a few years
- Understanding how the investment and clinical landscapes are evolving
- How has the field progressed in the last year and what kinds of challenges have come to the fore?

Janet Lynch Lambert
CEO
Alliance for Regenerative Medicine (ARM)

10.30am Morning Break & Networking

CONFERENCE DAY TWO **WEDNESDAY, FEBRUARY 24**

All times are in EST

CMC & ANALYTICAL
Aligning Regulatory Expectations with Current CMC Challenges

11.00am Gene Therapy CMC Strategies, Approaches & Lessons Learned
- What does the final CMC package need to look like?
- Beyond individual assays – how does the entire CMC strategy take shape and what are the unique challenges involved in gene therapy CMC approaches?
- Discussing the ever-evolving regulatory requirements in the field

Mauricio Umana, *Associate Director, Global Regulatory CMC Lead,* **Biogen**

11.30am Can a Platform Approach Address CMC Challenges Specific to CGT for Rare Disorders?
- Discussing technical challenges faced due to novelty of product modality, manufacturing process and limited prior experience
- Understanding the limited time for CMC development due to accelerated pathways
- Addressing the manufacturing experience due to small number of clinical batches

Speaker to be confirmed

MANUFACTURING SCALE-UP
Establishing Scalable Manufacturing Processes

11.00am Effective Approaches to Industrialize the Manufacturing of AAV Vectors for Gene Therapy Applications
- CMC competencies required for effective AAV product development
- Building internal vs. leveraging external capabilities
- Production platform choices and consequences
- Outlook for AAV manufacturing

Speaker to be confirmed, **Voyager Therapeutics**

11.30am State of Gene therapy Manufacturing
- Insights into the quality and purity of material generated by various manufacturing methods
- Investigating new developments to optimize the productivity of the platforms currently available
- Highlighting the approaches most amenable to scale-up and late stage development

Palani Palaniappan, *Chief Technology Officer,* **Aruvant**

CLINICAL
Understanding Product Differentiation in an Increasingly Crowded Therapeutic Landscape

11.00am Landscape Review of Gene Therapies for Rare Disorders
- A landscape review of the therapy and trials within gene therapy and rare disorder space
- With focus on some of the specific disease areas where gene therapies are seeing success and setbacks (blood disorders, neurology and ophthalmology disorders, etc.)
- The take at the later phase assets and how they will contribute to the future of gene therapy

Paulina Szymanska, *Senior Account Manager,* **Beacon Targeted Therapies**

11.30am What Must be Improved in AAV-Based Gene Transfer Technologies?
- Analyzing current limitations to AAV therapies
- Investigating novel technologies that optimise delivery
- Novel therapeutic applications in sight

Olivier Danos, *CSO,* **REGENXBIO**

COMMERCIAL
Articulating the Value of Gene Therapies to Overcome Market Access Challenges

11.00am Textbook vs. Reality: How the "Real World" Evaluates Gene Therapies
From an academic and clinical perspective, the use case for gene therapies seems open and shut. Yet, when we objectively evaluate current and potential gene therapies in development many questions loom large. Queries touch upon multiple areas - not just clinical, and economic, but also sensitive ethical and psychological dilemmas stakeholders might face. This presentation will highlight how to best recognize and address these questions through education and evidence generation to ensure optimal launch success.

Nandini Hadker, **Trinity**
Krista Perry, **Trinity**

11.30am Uncovering Creative Approaches to Generate Relevant Data to Support Access to these Therapies
- Understanding approaches to articulate the value of gene therapies to put you in a position to provide access to these therapies
- What are the stipulations to provide access to gene therapy products?
- Highlighting the importance of early evidentiary needs assessment and high-quality data collection to illustrate the benefits of one-time treatments to support reimbursement decisions

Doug Danison, *VP, Head of Access, Value & Evidence Strategy (AVES),* **bluebird bio**

CONFERENCE DAY TWO **WEDNESDAY, FEBRUARY 24**

All times are in EST

12.00pm **Talk Details To Be Confirmed**

12.00pm **Roadmap to Successful Commercialization**

With current increase in development and investment, gene therapy companies will be looking at manufacturing and commercialization of their therapies. In this presentation, we will present:

- Critical factors to successful commercialization
- Discuss what's needed for late phase and commercial manufacturing
- Discuss how to pick the right strategic partner CMO

Jessica Hilmoe, *Technical Sales Manager, Manufacturing & Operations,* **MilliporeSigma**

12.00pm **Speaking Position Reserved for the Jackson Laboratory**

The Jackson Laboratory
Leading the search for tomorrow's cures

12.00pm **Speaking Position Reserved for Invitae**

INVITAE

12:30pm Networking Lunch

CMC & ANALYTICAL
Evaluating and Guaranteeing the Quality of Raw Materials

1.30pm **Tackling Key Challenges Involving Raw & Starting Materials for Gene Therapy Manufacturing**

- Understanding the quality of raw materials required for gene therapy products
- Establishing effective standards and parameters to maintain the quality of raw materials
- How can you re-evaluate raw material strategies during scale-up to ensure they are appropriate?

Jim Richardson, *Senior Scientific Liaison,* **US Pharmacopeia**

MANUFACTURING SCALE-UP
Investigating the Key Factors Involved in Implementing Cost-Effective and Efficient Manufacturing Processes at Every Scale

1.30pm **Challenges of Manufacturing Gene Therapies into Commercial Realities**

- Overview of bluebird bio's efforts to advance gene therapies for severe genetic disorders and cancer immunotherapy
- Challenges faced in manufacturing cell therapies as they move toward commercial phase – with a particular focus on managing a network of CDMOs
- Approaches bluebird bio is implementing to overcome some of these challenges

Steven Goodman, *Head, Drug Product Manufacturing,* **bluebird bio**

CLINICAL
Channelling Patient Input for a More Successful Gene Therapy Program

1.30pm **Overcoming Barriers to Patient Recruitment for Clinical Trials**

- Realizing the need for earlier and better diagnosis
- Minimizing practical hurdles to reaching the clinic
- Clarifying eligibility of patients for trials – matching the right trial to the patient

Emily McGinnis, *Chief Patient Officer & Head of Government Affairs,* **Taysha Gene Therapies**

COMMERCIAL
Evaluating Rare Disease Business Models & Integrating the Patient Perspective in Affordability & Access Discussions

1.30pm **Rare Disease Portfolio: Financial Engineering Can Help Bring More Funding to Develop Orphan Drugs**

- The portfolio approach reduces volatility of investing in rare disease therapeutics
- Leveraged financing using structured debt can reduce risk and increase returns
- Increased funding can help more rare disease patients have access to much needed therapies

Nora Yang, *Chief Scientific Officer,* **Stratify Therapeutics**

CONFERENCE DAY TWO **WEDNESDAY, FEBRUARY 24**

All times are in EST

2.00pm **ROUNDTABLE SESSION: Transitioning Effectively to GMP Raw Materials**

- What does phase-appropriate development really mean in the context of raw materials?
- How can you re-evaluate raw material strategies during clinical process to ensure they are appropriate?
- Investigating the technical requirements at each stage of development

2.00pm **Technology Breakthroughs and Solutions in Plasmid and Viral Vector Manufacturing**

- Breakthrough clarification and harvest technology that can signal a paradigm shift in mAbs bioprocessing
- Advances in multiple disciplines, combining novel polymer technology, various filtration modes, hardware, systems and automated process management
- Potential benefits to productivity, product quality, yield and workflow efficiency will be discussed

Shelly Parra, *Director, Global Field Applications,* **Repligen**

2.00pm **Partnering with Patient Advocacy Groups: An Integrated Approach to Gene Therapy**

- Discussing when and how best to establish connections with patient advocacy groups
- Liaising with advocates in a way that maximises outcomes for all stakeholders
- Understanding the role patient advocacy groups play at multiple stages of the program

Emily Milligan, *Executive Director,* **Barth Syndrome Foundation**

2.30pm **Value-Based Agreements: In Theory vs In Practice**

- Articulating value of gene therapy to each stakeholder will be important regardless of reimbursement model
- Value-based models sound good in principle – how will this play out in practice?
- Will every gene therapy need a bespoke reimbursement model?

Mindy Cameron, *Consultant,,* **Little Hercules Foundation**

2.30pm Afternoon Refreshments & Networking

Involving the Patient Perspective in Gene Therapy Development & Commercialization

3.00pm **Emphasizing the Role of the Patient in Trial Design**

- Understanding what a meaningful outcome is for the patient
- Designing trials to minimise impact on patient everyday life
- Establishing meaningful endpoints using patient input

Jill Dolgin
Head of Patient Advocacy
AGTC

3:30pm **Incorporating the Patient Voice at Every Stage of the Program**

- Understanding patient views to explain their unmet needs to regulators for trial approval
- Strategies to allow optimal patient input for clinical programs
- Different approaches for different circumstances

Jayne Gershkowitz
Chief Patient Advocate
Amicus Therapeutics

4.00pm **Integrating Patient Preferences into Payment & Access Mechanisms for Transformative Gene Therapy Treatments**

- Bridging the gap between patients and payers
- Case study: How the Cystic Fibrosis Foundation is addressing cost, affordability and access
- Investigating the realities of patient access to high-priced treatments

Leslie Powell
Director, Policy & Advocacy
Cystic Fibrosis Foundation

4.30pm **Our Life...Our Fight. We need a cure for NF2**

- Patient's perspective on living with a progressing debilitating rare disease
- How patients and families are fighting back
- Novel therapies hope for NF2 (Neurofibromatosis type 2)

Gilles Atlan
VP
NF2 BioSolutions

5.00pm **PANEL DISCUSSION: How can Patient Advocacy Groups Work More Effectively with Payers?**

- Including the patient voice to educate and inform payers with real-life experiences from patient advocates
- Understanding the implications of reimbursement strategies on patients and the wider family
- Contrasting the discussions in indications with existing therapeutic options and those with a lack of treatment options

Peter Saltonstall
CEO
National Organization for Rare Disorders (NORD)

Kelly Maynard
President & Founder
Little Hercules Foundation

Katherine Dallow
VP, Clinical Programs & Strategy
Blue Cross Blue Shield of Massachusetts

Christian Rubio
Vice President, Strategic Advancement
Global Genes

5.30pm **Chair's Closing Remarks**

Chris Mason
CSO
AVROBIO

DISCUSSION DAY: THURSDAY, FEBRUARY 25

All times are in EST

9.00am **Chair's Opening Remarks**

Federico Mingozzi
CSO
Spark Therapeutics

Is Gene Therapy Really a Cure? Re-Dosing: The Elephant in the Room

9.15am **Understanding the Current Challenges of Immunogenicity and Re-Dosing in Gene Therapy**

- Exploring the challenges associated with administering AAV vectors
- Assessing the need and feasibility of re-dosing with current technologies
- Understanding the safety of re-dosing

Federico Mingozzi
CSO
Spark Therapeutics

9.45am **Mitigation of Immunogenicity to Enable Re-Dosing of AAV Vectors by Co-Administration with Tolerogenic ImmTOR Nanoparticles**

- Co-administration of ImmTOR nanoparticles with AAV inhibits the formation of anti-AAV antibodies and enables vector re-dosing
- In addition, admixing of ImmTOR to AAV has a beneficial first dose effect on enhancing transgene expression in the liver
- Clinical proof-of-concept has been established with ImmTOR combined with a highly immunogenic enzyme therapy

Kei Kishimoto
CSO
Selecta Biosciences

10.15am **Morning Refreshments**

Investigating Challenges of Immune Responses

11.00am **A Two-Fold Problem: Investigating the Challenges of Immune Responses to Transgenes**

- Understanding potential risk of developing immune responses to newly expressed proteins
- Comparing and contrasting immune responses to capsids and transgenes
- Exploring the role of Regulatory T cells in suppressing muscle inflammation and immunity.
- Immunoregulation versus immunosuppression during gene therapy

Armando Villalta
Assistant Professor
University of California

11.30am **Inhibitors in Hemophilia and Treatment Approaches for Eliminating Antidrug Antibodies with Gene and Immune Therapy**

- AAV liver gene transfer induces transgene tolerance mediated by Treg induction
- Immune tolerance is dependent on several factors such as AAV vector serotype, antigen expression level, transgene, and genetic background
- Adjunct immune suppression can complement AAV immune tolerance and prevent AAV capsid Nab

David Markusic
Assistant Research Professor of Paediatrics
IUPUI

12.00pm **Lunch & Networking**

DISCUSSION DAY: THURSDAY, FEBRUARY 25

All times are in EST

1.00pm **A Proactive Approach to Immunogenicity – Avoiding Immune Response**

- Understanding the importance of selecting the vector and administration route that de-risks immune response upfront
- Exploring how route of administration affects immunogenicity – is the eye really an 'immunoprivileged' organ?

Vibha Jawa
Director and Lead
Merck

1.30pm **Immunotherapeutic Interventions**

- Pre-existing vs. naïve anti-AAV immunity require different targeted interventions
- B-cell and antibody depletion are key for pre-existing immunity but may not be sufficient
- In naïve subjects: conditioning regimen and B/T -cell mediated immune tolerance induction
- Advanced immune monitoring strategies and experimental medicine trials are key for progressing immunotherapeutic interventions in patient populations

David Favre
Vice President, Translational Medicine
Asklepios BioPharmaceutical

2:00pm Afternoon Break

Analyzing the Best Approaches to Increase Likelihood of Re-Dosing

2.30pm **Exploring Immunosuppression as an Approach to Mitigate Immune Response**

- Effect of immunosuppression to prevent immune responses to the AAV capsid and the transgene
- Effect of immunosuppression to allow for repeated AAV dosing Effect of immunosuppression to manage pre-existing immunity

Manuela Corti
Assistant Professor
University of Florida

3.00pm **Panel Discussion: Comparing and Contrasting Immunosuppression and Novel Approaches**

- Discussing the most promising approaches to overcome immunogenicity
- Investigating which approaches are most applicable to different indications
- Recognizing the importance of novel technologies in the immunology space

Kei Kishimoto
CSO
Selecta Biosciences

Armando Villalta
Assistant Professor
University of California

Manuela Corti
Assistant Professor
University of Florida

Gabor Veres
VP, Head of Gene Therapy
BioMarin

3.45pm **Chair's Closing Remarks**

Federico Mingozzi
CSO
Spark Therapeutics

Close of the Post-Conference Discussion Day

WHO WILL YOU MEET?

> "The conference really brought the industry leaders together and provided an excellent opportunity to learn and monitor the pulse of the field"
>
> **Previous Attendee, Amicus Therapeutics**

Our focused, bespoke program brings together people and companies who are actively working in the gene therapy space to treat rare diseases.

Make the most of our integrated networking platform to build vital relationships for the future, and take part in valuable, in-depth discussions that will ultimately translate into more actionable insights for your own gene therapy pipelines.

95+ SPEAKERS

600+ ATTENDEES

200+ COMPANIES

Attendee Breakdown*

Attendee Seniority

- C Level 17%
- VP 19%
- Director 34%
- Other 17%
- Manager 8%
- Scientist 5%

Industry Breakdown

- Pharma 18%
- Biotech 50%
- Not for Profit 6%
- Academic 7%
- Contract Service Provider 12%
- Other 7%

Snapshot of Previous Attendees

Amicus Therapeutics, avexis, AVROBIO, axovant GENE THERAPIES, Biogen, BIOMARIN, bluebirdbio, Celgene, CSL Behring Biotherapies for Life™, Encoded THERAPEUTICS, Genentech, Johnson & Johnson, LogicBio THERAPEUTICS, MERCK, Neurocrine, novasep, NEUROGENE, Pfizer, PTC THERAPEUTICS, REGENERON, RENOVACOR, Retrophin, Sangamo THERAPEUTICS, SANOFI, SAREPTA THERAPEUTICS, SOLID BIOSCIENCES, Spark THERAPEUTICS, Takeda, ucb, ultragenyx pharmaceutical, uniQure, VERTEX, Voyager THERAPEUTICS

Statistics based on Gene Therapy for Rare Disorders 2019

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PARTNER WITH US

We provide limited opportunities for service providers to engage the world's leading gene therapy organizations.

This is your opportunity to influence the conversation in a rapidly growing landscape. There is an increasing appetite to address the key challenges within manufacturing, analytics, clinical development, regulation and commercialization.

However, it has recently become increasingly difficult to build relationships with new organizations, and our partners are working with us to build their business development pipelines efficiently. If you're looking to connect with the decision makers at pharma and biotech in gene therapy, this established meeting will provide you with a unique platform to showcase your capabilities and build your brand awareness.

Get in touch to explore how we can position your brand in front of this targeted audience, or schedule a demo of the platform.

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Top 3 Reasons to Attend

1. **SAVE** valuable time and resources by learning how the leading companies in the space have optimized manufacturing, clinical and commercial approaches
2. **GAIN** first-hand insights from the regulators and payers who will make the decisions that will directly influence the success of your gene therapy programs
3. **FORM** lasting connections by engaging directly with colleagues from the leading pharma and biotech companies actively developing gene therapies in an intimate environment

Drug Developer Pricing	Register & Pay By Friday, December 11	Standard Rate
FULL ACCESS PASS: 2 Day Conference + Workshop Day AND Discussion Day	$3797 (save $700)	$4097
2 Day Conference + Workshop Day OR Discussion Day	$2998 (save $600)	$3298
2 Day Conference	$2199 (save $500)	$2499
**Focus Evening	Free To Attend	

Standard Pricing	Register & Pay By Friday, December 11	Standard Rate
FULL ACCESS PASS: 2 Day Conference + Workshop Day AND Discussion Day	$4497 (save $700)	$4797
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2 Day Conference	$2899 (save $500)	$3199
**Focus Evening	Free To Attend	

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3 Easy Ways To Book

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Vice President - New Therapeutic Targets Discovery, UniQure
Gene Therapy for Neurological Disorders Europe Digital Attendee

"Very efficiently organized - considering it was virtual - excellent speakers."
CEO, Eyevensys
Gene Therapy for Ophthalmic Disorders Digital Attendee

"It was very informative and worked exceptionally well as a remote meeting."
Senior Director, Translational Medicine, AveXis
Gene Therapy for Neurological Disorders Europe Digital Attendee

"Very well organized and informative virtual conference!"
Senior Director - Regulatory Affairs, Adverum
Gene Therapy for Ophthalmic Disorders Digital Attendee

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
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Changes to Conference & Agenda:
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