Gene Therapy Analytical Development

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November 19-21, 2019 | Boston, MA

Optimizing Analytical Methods to Guarantee Safe, High Quality, Consistent & Efficacious Gene Therapy Products

Peter Marks
Director, CBER
FDA

Jeffrey Glenn
Lead, Analytical Sciences
Spark Therapeutics

Fengrong Zuo
Senior Director, CMC, Regulatory, Quality & Analytical Development
AveXis

Russell Goetze
Scientist
Bluebird Bio

Svetlana Bergelson
Director, Technical Development
Biogen

Qin Zou
Associate Research Fellow & Group Leader
Pfizer

"Best conference of the year for gene therapy companies to understand how our industry is tackling the challenges of pioneering the development of our advanced therapy products."

Benjamin Dewees, Sangamo Therapeutics

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WELCOME TO THE INAUGURAL GENE THERAPY ANALYTICAL DEVELOPMENT SUMMIT

The Gene Therapy Analytical Development Summit is the only conference solely dedicated to overcoming the unique analytical challenges encountered in the gene therapy space.

In the context of unprecedented gene therapy clinical progress and mounting regulatory scrutiny, the inaugural Gene Therapy Analytical Development Summit will unite large pharma and innovative biotechs to develop robust analytical tools to guarantee the consistency, quality and safety of gene therapy products.

Focused specifically on addressing the unique analytical challenges posed by gene therapy vectors, this technical scientific conference will unite the 'boots on the ground' scientists grappling with these challenges first-hand.

This is your opportunity to enhance your existing analytical methods and explore innovative novel tools to support safe and effective gene therapy development.

Optimize every stage of your analytical development:

Contract qPCR and ddPCR to Identify the Best Vector Titering Approach

Understand how to use titering methods to support analytical processes as well as determining dose, with insights from **Takeda**

Develop Phase-Appropriate Potency Assays

Investigate real-world case studies from **AveXis's** potency assay to support the approval of Zolgensma, to **Logic Bio's** potency assays for earlier stage gene therapies with unique genome integration characteristics

Learn About Alternatives to Variable & Outdated Infectivity Assays

Discover how **REGENXBIO** are utilizing relative infectivity as a reliable alternative to the historically variable TCID50 assay

Discern Empty, Full & Intermediate Capsids with Greater Clarity & Precision

Gain insights into the realities of analytical ultracentrifugation as a tool to provide direct quantification of empty, full and intermediate capsids, with insights from **Pfizer**

Utilize Next Generation Sequencing to Enhance Genome Characterization

Compare the NGS platforms that **Biogen** have utilized to discover how effectively they can align with increasingly stringent regulatory requirements.

YOUR EXPERT SPEAKERS

Svetlana Bergelson
Director, Technical Development
Biogen

Peter Marks
Director, CBER
FDA

Wei-Chiang Chen
Principal Scientist, Analytical
Flexion Therapeutics

Win Den Cheung
Associate Director, Analytical Development
REGENXBIO

Vivian Choi
Head of Gene Therapy Research, US, R&D
Takeda

Pete Clarner
Senior Associate Scientist, Gene Therapy Accelerator Unit
Biogen

Nathalie Clement
Associate Director & Associate Professor
Powell Gene Therapy Center, University of Florida

Lauren Drouin
Associate Director, Analytical Development
Logic Bio

Marina Feschenko
Principal Scientist
Biogen

Jeffrey Glenn
Lead, Analytical Sciences
Spark Therapeutics

Russell Goetze
Scientist
Bluebird Bio

Joseph Lee
Senior Director, Analytical Development, Gene Therapies
PTC Therapeutics

Xiaohui Lu
Director, Analytical Development
Ultragenyx

David Malliaros
Senior Director, Quality Control
Ultragenyx

Harald Messer
Scientist I, Manager, Analytical Development
AGTC

Patrick Starremans
Associate Director, Analytical Sciences & Head of Process Analytics
Voyager Therapeutics

Ales Strancar
Managing Director
BIA Separations

Sarah Sullivan
Scientist I
Biogen

Lawrence Thompson
Senior Principal Scientist
Pfizer

Yu Wang
Scientist II
Biogen

Zichuan Zhang
Senior Scientist
Sanofi

Qin Zou
Associate Research Fellow & Group Leader
Pfizer

Fengrong Zuo
Senior Director, CMC, Regulatory, Quality & Analytical Development
AveXis

PRE-CONFERENCE WORKSHOP DAY
TUESDAY, NOVEMBER 19

WORKSHOP A

8:00-11:00

Enhancing Analytical Comparability Strategies to Support Every Stage of Development

Whatever stage of development you're currently at, progressing to the next phase and adapting your analytical strategy is a key challenge you will need to navigate. This is all in the context of increasing regulatory scrutiny and uncertainty over the analytical and comparability strategies required to satisfy the regulators.

Attend this workshop to discuss these topics in more detail:

- Dissecting the transitions between early and late stages
- Developing effective bridging studies
- Dispelling uncertainties around the changes that can be made to processes while maintaining analytical comparability to satisfy regulatory agencies
- Understanding how analytical methods need to evolve and improve as development reaches later stages

Workshop Leader:

David Malliaros
Senior Director, Quality Control
Ultragenyx

WORKSHOP B

11:30-14:30

Discussing How to Develop Accurate Assays to Measure Residual Impurities

Analyzing both DNA and protein impurities is a key aspect of gene therapy analytical investigation. It's clear that there are numerous challenges involved in this analysis, that limit the potential to establish methods that capture the extent and diversity of impurities present.

This workshop represents an opportunity to discuss these challenges and learn about:

- Insights into the use of an HSV platform and the assays utilized
- Understanding approaches to measure DNA and plasmid residuals and also impurities from the HSV helper virus system
- Investigating transferable, cross-platform insights
- Discussing standardization and how data and results can be shared across organizations in the field

Workshop Leader:

Nathalie Clement
Associate Director & Associate Professor
Powell Gene Therapy Center, University of Florida

WORKSHOP C

15:00-18:00

Investigating Outsourcing Strategies to Utilize Internal and External Resources Effectively

Establishing mutually beneficial collaborations with external organizations is a key factor in long-term success. Given the complexity of gene therapy analytics, establishing these relationships is a complex task and decisions around when as well as what to outsource are key. This workshop will provide an opportunity for in-depth discussions from those who have been intimately involved in this aspect of this field.

Attend this workshop to discuss these topics in more detail:

- Internal vs External? Understanding the factors influencing decisions to outsource of invest in improving internal capabilities and how this situation is evolving in the field
- Highlighting the key criteria to assess in selecting an external partner organization
- Improving the tech transfer process: how to transfer key information successfully to maintain efficiency and safety
- Describing differences in the requirements for outsourcing small-scale upstream processes to running GMP testing

Workshop Leader:

Lauren Drouin
Associate Director, Analytical Development
Logic Bio

CONFERENCE DAY ONE - WEDNESDAY, NOVEMBER 20

7:30 **Registration, Coffee & Networking**

8:20 **Chair's Opening Remarks**

Svetlana Bergelson
Director, Technical Development
Biogen

ALIGNING INDUSTRY LEADERS AROUND REGULATORY EXPECTATIONS

8:30 **FDA Perspective on Analytical Regulatory Expectations**

- Provide insight in to the type of analytical data required to support agency licensure
- Present case studies reflective of specific requirements reflective of recent regulatory experience
- Share the need for adaptation to the specific and unique nature of the gene therapy program – how do expectations differ between indications and scales of manufacturing
- Review the validation necessary for new technologies – methods, instruments and software
- Offer insight into the current and future regulatory paradigms for gene therapy manufacturing

Peter Marks
Director, CBER
FDA

9:00 **Presentation Details to be Confirmed**

Fengrong Zuo
Senior Director, CMC, Regulatory, Quality & Analytical Development
AveXis

9:30 **Speed Networking & Morning Refreshments**

11:00 **Analytical Methods: A Path to More Efficient Processes & Safer Gene Therapy Products**

- A non-affinity AAV manufacturing process to manage formation of aggregates and result in extra low DNA contaminants will be presented
- Rapid and sensitive analytics for in-process control of AAV manufacturing with focus on HPLC and different in-line detectors will be shown.
- Qualitative and quantitative information can be obtained in near real-time to assist with process decision-making
- New ultra-sensitive DNA assay showing major AAV-associated DNA contamination will be introduced

Ales Strancar
Managing Director
BIA Separations

11:30 **Panel Discussion: Evaluating the Analytical Strategies of the Leading Companies in the Field**

This interactive panel discussion will bring together the leading companies in the field to discuss key takeaways from pioneering gene therapy programs. Topics to be covered include:

- Investigating common analytical challenges encountered across various programs in different indications and with different specifications
- Balancing the complexities of analytical requirements with the drive to progress therapeutics to market
- Establishing collaborative relationships with other departments within the organization
- Lessons learned from the recent approvals of gene therapy products – how has feedback from agencies changed and shaped analytical strategies moving forward?

Fengrong Zuo
Senior Director, CMC, Regulatory, Quality & Analytical Development, **AveXis**

Jeffrey Glenn
Lead, Analytical Sciences
Spark Therapeutics

Russell Goetze
Scientist
Bluebird Bio

Peter Marks
Director, CBER
FDA

12:15 **Lunch & Networking**

DEVELOPING EFFECTIVE AND CLINICALLY RELEVANT POTENCY ASSAYS

13:45 **Potency Assessment for Novel AAV-Based Drugs: Exploration of Different Biological Activities & Stability Indicating Properties of the Assays**

- There are three major types of in-vitro analytical assays for assessment of biological activity of AAV-based therapeutics: infectivity, target protein expression, and functional potency
- It is important to understand what information each assay can provide and how it can be used for process development and drug characterization
- Changes in AAV vector structure affect three biological assays in a different manner
- What strategy can we use if MOA is not clear?

Marina Feschenko
Principal Scientist
Biogen

CONFERENCE DAY ONE - WEDNESDAY, NOVEMBER 20

14:15 **Developing a Potency Assay for GeneRide Products: an AAV-based Genome Editing Platform**

- The Gene Ride platform utilizes the natural process of homologous recombination to achieve targeted genome editing without the use of exogenous nucleases.
- Current guidance on potency assay development will be discussed, including specific obstacles for gene therapy products.
- Developing a potency assay for GeneRide vectors demonstrates additional challenges including low levels of integration which requires highly sensitive detection methods. Surrogate methods to investigate biological activity will be described.

Lauren Drouin
Associate Director, Analytical Development
Logic Bio

14:45 **Interactive Roundtable Discussions: Deep Dives into Key Existing & Emerging Technologies**

Participate in a focussed roundtable discussion to discuss key analytical technologies in an intimate environment. Each table will be led by an industry leader with experience in using the relevant methodology, so this is your opportunity to have your burning questions answered and benchmark your approach with your colleagues in the field. Roundtables will discuss the use of the following approaches

qPCR and ddPCR

Xiaohui Lu
Director, Analytical Development
Ultragenyx

Chromatography

Wei-Chiang Chen
Principal Scientist, Analytical
Flexion Therapeutics

Analytical Ultracentrifugation

Qin Zou
Associate Research Fellow & Group Leader, **Pfizer**

Next Generation Sequencing

Sarah Sullivan
Scientist I
Biogen

15:30 **Afternoon Refreshments & Scientific Poster Session**

The scientific poster session is an ideal opportunity to communicate your new results and expertise to a niche audience of industry experts, get feedback from peers and colleagues in the industry and learn how others in the field have been tackling similar challenges.

PIONEERING INNOVATIVE METHODOLOGIES TO EVALUATE CAPSID & GENOME QUALITY

16:00 **Strategies & Technologies for AAV Genome Characterization**

- Understanding the inherent complexities to AAV genome study
- Overview of traditional techniques for genome characterization and their limited capacity to meet regulatory requirements
- Comparison and evaluation of next generation sequencing platforms and their accompanying data analysis

Sarah Sullivan
Scientist I
Biogen

16:30 **Analytical Ultracentrifugation: A Critical & Versatile Tool to Evaluate Gene Therapy Viral Vectors**

- Analytical Ultracentrifugation is the only tool to provide direct quantification on empty, intermediate and full viral capsids
- Analytical ultracentrifugation using multi-wavelength detection offer greater confidence in characterizing the particle content
- Various forms of analytical ultracentrifugation can provide additional information that other separation techniques cannot

Qin Zou
Associate Research Fellow & Group Leader
Pfizer

17:00 **AAV Capsid Purity: the Control Strategy & the Evaluation of CE-SDS with LIF Detection**

- Insights into the current strategies and gaps for determining capsid protein purity of AAV gene therapy candidates
- Providing an overview of novel labeling approaches to enable CE-LIF detection of AAV capsid proteins
- CE-LIF could be a QC-friendly method for routine monitoring of capsid purity compared with other CE-based analysis

Zichuan Zhang
Senior Scientist
Sanofi

17:30 **Chair's Closing Remarks**

Svetlana Bergelson
Director, Technical Development
Biogen

17:45 **End of Conference Day One**

CONFERENCE DAY TWO - THURSDAY, NOVEMBER 21

8:20 **Chair's Opening Remarks**

Svetlana Bergelson
Director, Technical Development
Biogen

IMPROVING CURRENT ANALYTICAL METHODS TO GIVE MORE CONSISTENT & ACCURATE RESULTS

8:30 **Analytical Development Strategies for AAV Gene Therapy programs**

- Considerations for development of analytical assays
  - Assay throughput: In-process vs Release
  - To qualify or not to qualify?
  - In-house use vs outsourcing models
- Development strategies for cell-based potency assays
  - Phase appropriate development
  - (case study)
- Switching gears
  - Adapting to program and assay changes
  - (case study)

Patrick Starremans
Associate Director, Analytical Sciences & Head of Process Analytics
Voyager Therapeutics

9:00 **Overview of Challenges in rAAV Custom Assay Design**

- Understanding approaches such as rational design for improved risk assessment on key project systems in ADA
- Recognizing sources of inherent variability in potency assay development for cell & gene therapy products
- Assimilating exploratory and bio-pharmaceutics development in potency assay work with complex MOA

Harald Messer
Scientist I, Manager, Analytical Development
AGTC

9:30 **Sharing the Various Molecular Testing Components Required to Support Product Characterization**

- Investigating the components required to support analytical development
- Beyond ID and titer – understanding the characteristics that need to be defined to work towards product safety
- Discussing the factors influencing decisions on in-house and external development

Joseph Lee
Senior Director, Analytical Development, Gene Therapies
PTC Therapeutics

10:00 **Morning Refreshments & Networking**

ENSURING ACCURACY IN VECTOR GENOME TITERING TO ESTABLISH CORRECT & REPRODUCIBLE DOSING

11:00 **Contrasting qPCR & ddPCR Methods for Vector Titering**

- What's the best method to use? Investigating the advantages and disadvantages of qPCR and ddPCR at various stages of development
- How to use titer methods to support the process as well as releasing the drug
- Understanding how to successfully transition from early stage qPCR to ddPCR for commercial material: how to address differences through comparability studies and meet regulatory expectations

Vivian Choi
Head of Gene Therapy Research, US, R&D
Takeda

11:30 **PCR-based Approaches for Determining AAV Genome Titer in Support of Gene Therapy Development**

- The criticality of genome titer assays at all different stages of development
- The advantages and disadvantages of ddPCR and qPCR for gene therapy vector titering and development support
- Strategies for method crossover and when to apply a platform

Pete Clarner
Senior Associate Scientist, Gene Therapy Accelerator Unit
Biogen

12:00 **Lunch & Networking**

CONFERENCE DAY TWO - THURSDAY, NOVEMBER 21

BEYOND AAV: INVESTIGATING ANALYTICAL STRATEGIES TO EVALUATE PLASMIDS & LENTIVIRAL VECTORS

13:00 **Plasmid DNA Control Strategy Drug Substance-Product vs Critical Raw Material**
- Specifications, Comparability, Potency assays, Stability, etc.
- Method & Process Validation
- Safety Risk vs Business Risk
- Current Regulatory Guidance

Lawrence Thompson
Senior Principal Scientist
Pfizer

13:30 **Panel Discussion: Contrasting Analytical Requirements for AAV & Lentiviral Vectors**
- Discussing the relative advantages and disadvantages of lentiviral and AAV vector approaches in a variety of indications
- Understanding how analytical strategies differ in the context of different viral vectors
- Case studies detailing similarities and differences in the analytical approaches required for the analysis of different vectors

Harald Messer
Scientist I, Manager, Analytical Development
AGTC

Russell Goetze
Scientist
Bluebird Bio

14:00 **Afternoon Refreshments & Networking**

IMPROVING CURRENT INFECTIVITY ASSAYS TO GIVE MORE CONSISTENT RESULTS

14:30 **A Novel Approach to Measuring AAV Infectivity with ddPCR Technology**
- Design, development and qualification of a new AAV infectivity assay by ddPCR
- Head-to-head comparison with the existing TCID50 method
- Discussing pros and cons of the new infectivity assay

Yu Wang
Scientist II
Biogen

15:00 **Relative Infectivity as a Reliable Alternative to the TCID50 Assay**
- Understanding the limitations of the TCID50 infectious titer assay
- Advantages of the relative infectivity method over the TCID50 assay
- Utility of the relative infectivity method in a variety of applications to support early development

Win Den Cheung
Associate Director, Analytical Development
REGENXBIO

15:30 **Chair's Closing Remarks**

Svetlana Bergelson
Director, Technical Development
Biogen

15:45 **Close of Conference**

"This is "the conference" to attend for professionals that are already involved or about to embark in the gene therapy space. All of the relevant experts in their field are represented and available to share their knowledge and experience. A true educational and networking event!"

Bill White, Senior Director, Market Access, **AveXis**

"The conference really brought the industry leaders together and provide an excellent opportunity to learn and monitor the pulse of the field."

Xiaoyang Wu, Director, Translational Science, **Amicus Therapeutics**

"Extremely informative. Case study presentations were very interesting"

Spyros Papapetropoulos, Consultant, Neurology, **Massachusetts General Hospital**

WHO WILL YOU MEET?

Uniting experts from a range of key departments, including:

- ANALYTICAL DEVELOPMENT
- QC/QA
- PROCESS DEVELOPMENT
- REGULATORY

Rather than analytical challenges representing a small sub-section of a larger conference, these issues are the sole focus of this event.

This niche content and focused audience facilitates **more in-depth and valuable conversations,** and at the end of the day **more actionable insights for you to take away.**

INDUSTRY BREAKDOWN*

Pharma	20%
Biotech	46%
Not-for-Profit	2%
Academic	6%
Service Provider	18%
Other	8%

GENE THERAPY EVENT SERIES ATTENDEES

Allergan, Amicus Therapeutics, avexis, AVROBIO, axovant GENE THERAPIES, BIOMARIN, bluebirdbio, REGENERON, GILEAD, Orchard therapeutics, Pfizer, PTC THERAPEUTICS, MEIRAGTx, Johnson & Johnson, REGENXBIO, SANOFI, SAREPTA THERAPEUTICS, Shire, SOLID BIOSCIENCES, ultragenyx gene therapy, uniQure

Statistics based on Gene Therapy for Rare Disorders Europe 2018

PARTNER WITH US

After decades of unfulfilled potential, the commercial reality of the gene therapy field is rapidly taking shape.

However, the leading drug developer organizations in this space are telling us that developing effective analytical strategies is a major bottleneck for them, and that they have little visibility into which contract service providers and technology providers have the analytical experience and specific technologies to meet their demands.

Analytical and CMC leaders at the leading companies in the gene therapy field will unite this November at the Gene Therapy Analytical Development Summit, actively seeking to collaborate with organizations that can help them solve their analytical challenges.

If you have capabilities that would benefit our audience, Gene Therapy Analytical Development will provide a unique platform to showcase your solutions, generate new business leads and source opportunities for partnerships.

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

BIA SEPARATIONS
LEAD PARTNER

BIA Separations, with its 20 years of experience in DSP, is fully dedicated to the development of best-in-class analytical and preparative chromatographic solutions within the Gene- and Cell Therapy space.

Convective Interaction Media (CIM®), utilized in hundreds of clinical batches, are setting the standard of manufacturing highly complex ATMPs such as AAVs, pDNAs, mRNAs, Viral Vaccines and Exosomes, today. Choosing BIA Separations as a CRO provides you with the best assurance, not only of quality processing, but also the process controls and high product quality you need. In time.

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ACF Bioservices is the only analytical CRO exclusively dedicated to supporting CGT. Our CGT bioanalytical services include de novo assay development through Qualification, Validation, and GMP-compliant release testing. We have experience with immunotherapies – ex-vivo, in-vivo, autologous, allogeneic and non-vector mediated therapies. ACF's nonclinical in-vivo services (GLP or Non-GLP) can be performed for proof of concept, efficacy, toxicity, biodistribution, and shedding. Our in-vitro services (Non-GLP, GLP, GMP) can Develop, Qualify and Validate relative potency assay, stability assay, neutralization antibody assay, vector infectivity (TCID50). ACF is FDA inspected and AAALAC accredited..

www.acfbio.com

Nathan Wilgoss
Senior Partnerships Director
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READY TO REGISTER?

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Package Details	Register & pay by Friday, August 23	Standard Price
Conference + 3 Workshops	**$3,496** (SAVE $1,200)	**$4,396** (SAVE $300)
Conference + 2 Workshops	**$2,997** (SAVE $1,100)	**$3,897** (SAVE $200)
Conference+ 1 Workshop	**$2,498** (SAVE $1,000)	**$3,398** (SAVE $100)
Conference only	**$1,999** (SAVE $900)	**$2,899**
1 workshop only	**$599**	

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3 delegates

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

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