3rd Annual

# Gene Therapy Immunogenicity

**October 18-20, 2022**
**Boston, MA**

REGISTER & PAY BY FRIDAY, SEPTEMBER 23 & SAVE UP TO $300

# Optimizing Preclinical & Clinical Immunogenicity Assays & Strategies

## 2022 Speakers Include:

**Olga Uspenskaya**
Vice President, Clinical Development
**Prevail Therapeutics**

**Joshua Friedman**
Head Translational Science
**Spark Therapeutics**

**Steen Klysner**
Chief Executive Officer
**Amarna Therapeutics**

**Lillian Falese**
Associate Director, Bioanalytical Development
**Spirovant Sciences**

**Priya Chockalingam**
Vice President & Head of Clinical Bioanalytics & Translational Sciences
**Beam Therapeutics**

**William Quinn**
Director, Immunology
**Kriya Therapeutics**

**Jean-Philippe Combal**
Co-Founder & Chief Executive Officer
**Vivet Therapeutics**

**Katherine Block**
Director, Biomarker Sciences
**Sangamo Therapeutics**

**Roland Herzog**
Director - Gene, Cell Therapy Program
**Indiana University School of Medicine**

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# WELCOME TO THE 3RD ANNUAL GENE THERAPY IMMUNOGENICITY SUMMIT

## Overcoming Translational Hurdles, Improving Immunology Assays & Optimizing Clinical Strategies for Mitigating Adverse Immune Responses

**Overcoming the safety and efficacy challenges associated with immunogenicity of gene therapies is undoubtedly the fields biggest challenge. The ability to better modulate, predict and measure immune responses are critical steps for drug developers looking to progress their pipelines, thus now more than ever biopharma are maximising investment into immunogenicity research.**

The **3rd Gene Therapy Immunogenicity Summit** is the industry's leading forum enabling you to overcome immunity to viral vectors, understand how immune-modulatory approaches could enable re-dosing and get to grips with the reality of regulatory expectations around immunological assays.

Incorporating insights from leading immunological, bioanalytical and translational experts in the clinical and pre-clinical setting, this is your opportunity to gain extensive technical information on the contrasts between immunogenicity encountered across a range of tissues and administration routes, how models can be enhanced to enable comparative immunology between species and what the latest clinical data tells us about how to adapt existing approaches to address and manage immunogenicity.

Join **100+ fellow gene therapy immunogenicity experts** to learn from each other, make new connections in person and share your research as you seek new and improved ways of predicting immunogenicity in the clinic to ultimately ensure the safety and efficacy of gene therapies.

### Get to grips with best practice for companion diagnostic design, regulatory expectations and partnership validation

Hear from **Sangamo Therapeutics** and **Spark Therapeutics** as they deliver an exclusive discussion based session giving you all the information you need to kickstart companion diagnostic development

### Join our "Industry Leaders" plenary panel discussion as they address the realities, challenges and potential alternatives to high dose viral vector delivery

Join leaders from **Selecta Bio, Sana Bio, Vivet Pharma** and **Rocket Pharma** in an open discussion dissecting all things dose related

### Explore the latest understandings and research on the immunogenicity of both the payload and vector in the fields of gene therapy and gene editing

Hear from **Modalis Therapeutic**s and **Baylor College of Medicine** as they dissect their latest research and better understand how your product is evoking any immune response

### Learn best practice strategies in assay development and validation to better measure and predict immmune response

Hear from **Pfizer Spark Therapeutics & Sangamo Therapeutics** as they discuss their strategies to develop clinical enrollment assays to detect pre-existing Nab's and novel predictive assays for product characterization

### Understand how to develop an industry leading immunogenicity risk assessment for AAV based gene therapies

Discover how **Roche** and **Beam Therapeutics** developed their immunogenicity mitigation strategy including which criteria to account for and which assays are available to evaluate risks

# WHAT'S NEW FOR 2022

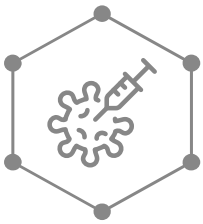
The fields understanding of immunology and virology in the context of gene therapy delivery continues to expand and so to does the content within this meeting. Take a look below and get stuck into some of our most highly anticipated sessions at this year's summit.

**Workshop: Companion Diagnostic Development for Gene Therapies**

Jump into this discussion based session led by **Sangamo** and **Spark Therapeutics** as they guide you through development hurdles including development and submission, CRO validation, regulatory interactions and cost expectations of CDx development

**Industry Leaders Panel: High Dose Delivery**

Hear from some of the industries leading voices from **Selecta Bio, Sana Bio, Vivet** and **Rocket Pharma** as they dissect the realities and challenges of high dose vector delivery and potential alternative strategies



**Workshop: Immune Modulation & Suppression Strategies**

Uncovering all things immune modulation this session lead by **Roche, Wistar** and **Kriya Therapeutics** will provide an in depth overview of current immune modulation best practices and how innovation in the space is creating more effective and targeted products

**Conference Session: Better Understanding Cellular Immunity & its Role in Immunogenicity**

Hear from **Roche** and **Director of Gene Therapy** at **Indianna University** as they disect the latest understanding of innate immune signalling pathways, the role of antigen presenting cells and complement and how such responses can be managed

**Conference Session: Better Understanding Immune Responses to Gene Editing**

Hear from **Modalis Therapeutics** and **Baylor College of Medicine** as they discuss anti cas-9 immunity, what this means for long term expression alongside strategies to prevent gene edited hepatocytes

## NEW CONTENT: PRE-CLINICAL TRACK

Our brand new pre-clinical track is designed to provide you with everything you need to navigate your biggest non-clinical hurdles. Uncover novel *in-vivo* models, explore immune profiling methodologies and better understand cellular immunity in the non-clinical setting by hearing from **Spirovant**, **Indiana University**, **Amarna, Roche** and more

## NEW CONTENT: CLINICAL TRACK

With the safety and efficacy of gene therapy products under more scrutiny than ever before, it has never been more pivotal to better understand and manage clinical manifestations of immunogenicity. This track is your one stop shop to better monitor and modulate immunogenicity in the clinic, examine the effects of administration route and uncover the role of complement by hearing from **Rocket Pharma**, **Beam Therapeautics**, **Adverum**, **Ask Bio** and more

## NEW CONTENT: IMMUNOGENICITY & ITS IMPLICATIONS FOR SAFETY

We are set to gather industry leaders from **Selecta Bio**, **Sana Bio**, **Modalis Therapeutics**, **Prevail Therapeutics**, **Roche**, **Spark Therapeutics** and more across panels, presentations and workshops to dissect the realities of safety concerns associated with immunogenicity alongside strategies to measure and modulate such immune responses to develop safer products

# YOUR EXPERT SPEAKERS

**Anna Majowicz**
Preclinical Immunology Lead
**Spark Therapeutics**

**Allison Keeler-Klunk**
Assistant Professor
**University of Massachusetts Medical School**

**William Lagor**
Associate Professor, Molecular & Cellular Biology
**Baylor College of Medicine**

**Roland Herzog**
Director - Gene, Cell Therapy Program
**Indiana University School of Medicine**

**Rebecca Xicluna**
Post Doctoral Fellow
**Roche**

**Boris Gorovits**
VP, *IN VITRO* Pharmacology, Biomarker Discovery and Bioanalysis
**Sana Biotechnology**

**Olga Uspenskaya**
Vice President, Clinical Development
**Prevail Therapeutics**

**Jonathan Schwartz**
Chief Medical Officer
**Rocket Pharmaceuticals**

**Joshua Friedman**
Head, Translational Science
**Spark Therapeutics**

**Rajakumar Mandraju**
Principal Scientist
**Modalis Therapeutics**

**Dongsheng Duan**
Professor
**University of Missouri School of Medicine**

**Kei Kishimoto**
Chief Scientific Officer
**Selecta Biosciences Inc.**

**Steen Klysner**
Chief Executive Officer
**Amarna Therapeutics**

**Hildegund Ertl**
Professor, Vaccine & Immunotherapy Center
**The Wistar Institute**

**Josh Holter**
Scientist
**Sangamo Therapeutics**

**Laura Salazar-Fontana**
**Independent Consultant**

**Lillian Falese**
Associate Director, Bioanalytical Development
**Spirovant Sciences**

**Priya Chockalingam**
Vice President & Head, Clinical Bioanalytics & Translational Sciences
**Beam Therapeutics**

**William Quinn**
Director, Immunology
**Kriya Therapeutics**

**Jean-Philippe Combal**
Co-Founder & Chief Executive Officer
**Vivet Therapeutics**

**Hélène Haegel**
Principal Scientist
**ImmunologyRoche**

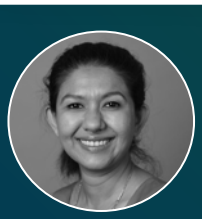
**Katherine Block**
Director, Biomarker Sciences
**Sangamo Therapeutics**

**Liching cao**
Director, Bioanalytical Operations
**Sangamo Therapeutics**

**Shari Gordon**
Senior Director, Immunology
**AskBio**

**Jatin Narula**
Principal Scientist, Biomedicine Design
**Pfizer**

# YOUR EXPERT SPEAKERS



**Moanaro Biswas**
Assistant Professor, Research
**Indiana University**

**Roberto Calcedo**
Vice President, Preclinical & Immunology
**Affinia Therapeutics**

**Ashlyn Bassiri**
Vice President, Immunology
**Kriya Therapeutics**

**Inderpal Singh**
Immunogenicity Prediction Lead
**Spark Therapeutics**

**Piet van der Graaf**
Senior Vice President, Head of Quantitative Systems Pharmacology
**Certara**

## Testimonials from last year's attendees:

"The Gene Therapy Immunogenicity Summit is an extremely important annual forum for new discoveries in Immunogenicity relating to Gene Therapy"

**Director, Adverum Bio**

"Will apply lessons imparted from this conference to my future sponsored clinical research"

**SVP, Excision Bio**

# PRE-CONFERENCE WORKSHOP DAY - TUESDAY, OCTOBER 18



## WORKSHOP A

8.00 - 11.00 AM

### Companion Diagnostics for AAV Gene Therapies 101

When used with gene therapy, companion diagnostics (CDx) can help inform treatment decisions. Thus, the identification of appropriate CDx has been proposed in multiple guidelines relevant to gene therapy. CDx are often in vitro diagnostic devices that provide information essential for safe and/or effective use of a corresponding drug or biological product. CDx can help identify patients who are likely to benefit from therapy or those likely to experience treatment-related adverse events. These tools may facilitate the monitoring of treatment response, enabling healthcare providers to adjust therapy and achieve improved safety or effectiveness. Current regulatory guidance recommends the development of CDx assays for gene therapy and were updated to use CDx not only for safety but for efficacy as well.

**In light of this, the workshop will set out to cover:**

- Introduction to CDx and when/why it is needed
- FDA Guidance for Gene Therapy (Disease Dx vs predictive biomarker for patient selection)
- Anti-AAV pre-existing Ab as a predictive biomarker
- Formats of pre-existing Abs assays (NAb, TAb)
- Timeline/development considerations for each assay
- CDx Assay development – overview of the process and key parts
- Best practice for establishing a partnership with a CRO for your companion diagnostic
- Impact of the regulatory environment

**Josh Friedman**
Vice President, Head of Translational Medicine
**Spark Therapeutics**

**Katherine Block**
Director Biomarker Sciences
**Sangamo Therapeutics**

## WORKSHOP B

11.30 - 2.30 PM

### Pre-Existing Immunity & Clinical Trial Eligibility

Clinical trial eligibility is a non-standardized enrolment criteria leveraged by some but not all biopharma across in-human trials. Most humans have varying levels of natural immunity to the AAV viral vector most commonly used in the gene therapy space and thus this can pose a potential challenge for drug safety and efficacy. As a result, many clinical trials patients are screened for existing immunity to the corresponding gene therapy product and a decision is made by the sponsor as to whether they are able to join the trial. As clinical trial eligibility is non-standardized and in some cases not used at all, there is a great deal of variability and associated challenges that come with the decision to set eligibility at certain levels, or not set one at all.

**As such this workshop will set out to address the following themes:**

- Correlating pre-screening of patients with clinical relevancy
- Nab or Tab approach for clinical trial eligibility criteria
- Exploring Peptide libraries for immune response screening
- Standardizing immune response testing: what level of antibody presence should be deemed as too high?
- Preclinical best practices to determine antibody thresholds

**Joshua Holter**
Bioanalytical & Translational Scientist
**Sangamo Therapeutics**

# PRE-CONFERENCE WORKSHOP DAY - TUESDAY, OCTOBER 18



## WORKSHOP C

3.00 - 6.00 PM

### Mitigating Immune Response: Immune Modulation & Suppression Strategies

Immunogenicity mitigation strategies largely take the form of either immunosuppressive regimes or removal of antibodies via mutagenesis or plasmapheresis. As gene therapy products are significantly immunogenic in nature, biopharma typically always need some kind of immune mitigation strategy to be implemented in order to meet required levels of safety and efficacy. With technological advances and a better understanding of immunogenic response, mitigation strategies have evolved to become more targeted and effective. Despite this, there still lies a large number of challenges for biopharma to address.

**This workshop will set out to address challenges including:**

- Moving away from broad hammer type approaches - how can we make immunosuppressive tailored tolerance to be specific to the capsid and the transgene?
- Uncovering novel immunosuppressive compounds or molecules that allow for more immediate modulatory effects
- Evaluating the suitability and success of current immunosuppresion agents in the context of gene therapy
- Evaluating current approaches and their comparative success for reducing the effects of neutralizing antibodies
- Novel mitigation strategies: Can engineering regulatory T cells suppress immune responses to factor 8?
- How effective can mutagenesis and plasmaphereses approaches be given they don't address innate immunity or T-cell response?

**Hildegund Ertl**
Professor Vaccine & Immunotherapies Centre
**The Wistar Institute**

**Helene Haegel**
Principal Scientist Immunology
**Roche**

**William Quinn**
Director Immunology
**Kriya Therapeutics**

"Inspiring virtual meeting allowing efficient learning about the most recent advances in Gene Therapy Immunogenicity while still having interactions with peers"

**Roche, 2021 Attendee**

"Excellent overview of the current understanding of AAV immunogenicity and new directions to mitigate it"

**REGENXBIO, 2021 Attendee**

"It was great to have a conference focused on analytical challenges for testing immune markers and modulating immune responses to gene therapies"

**Kriya Therapeutics, 2021 Attendee**

# CONFERENCE DAY ONE - WEDNESDAY, OCTOBER 19

8.50 **Chair's Opening Remarks**

## EXAMINING DOSE SIZE & DOSING STRATEGIES IN THE CONTEXT OF IMMUNE RESPONSE

9.00 **Addressing the Multifaceted Nature of AAV Immunogenicity**

- Immunogenicity of AAV is complex and challenging, involving elements of innate, humoral and cellular immunity that can adversely impact patient eligibility, safety, efficacy, durability of treatment, and ability to re-dose
- Mitigation strategies should account for all aspects of immunogenicity
- Discussing data on the effects of ImmTOR tolerogenic nanoparticles on innate, CD8 T cell, and antibody responses to AAV and combination strategies to address high dose toxicity

**Kei Kishimoto**
CSO
**Selecta Bio**

9.30 **Industry Leaders Panel Discussion: Addressing High Dose AAV Delivery**

- Have we reached the highest dose for gene therapy yet?
- Exploring dosing strategies: "One and Done" or "Lower & Slower"?
- What impact would a cumulative vector genome delivery approach have on safety and efficacy?
- Moving away from one and done: what does best practice look like for dosing strategies?
- Which role does complement play in the context of high dose gene therapy delivery

**Boris Gorovitz**
VP *IN VITRO* Pharmacology, Biomarker Discovery and Bioanalysis
**Sana Biotechnology**

**Kei Kishimoto**
Chief Scientific Officer
**Selecta Bio**

**Jean-Philippe Combal**
Co-Founder & Chief Executive Officer
**Vivet Pharma**

**Jonathan Schwartz**
Chief Scientific Officer
**Rocket Pharma**

10.30 **Speaking Position Reserved for Precision Medicine**

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11.00 **Speed Networking**

This session is the ideal opportunity to get face-to-face time with many of the brightest minds working in the gene therapy field and establish meaningful business relationships to pursue for the rest of the conference

11.30 **Morning Break & Refreshments**

## PRE-CLINICAL TRACK

### The Quest to Source & Leverage Pre-Clinical Models That More Accurately Reflect the Human Immune System

12.00 **Immune Response to AAV CRISPR Therapy for Duchenne Muscular Dystrophy**

- Examining immunogenicity to AAV based CRISPR therapy
- Modelling immune response in DMD model

**Dongsheng Duan,** Professor, **University of Missouri School of Medicine**

## CLINICAL TRACK

### Examining Clinical Manifestations of Immunogenicity

12.00 **Characterizing the Immunogenicity of AAV8 Empty Capsids in Healthy Volunteers**

- Empty Capsids are immunogenic inducing robust adaptive responses in seronegative healthy volunteers
- CD4+ T cell help is a major contributor to the overall immune response
- Empty Capsids can be used as a model antigen to evaluate the efficacy of Immune modulatory strategies in development

**Shari Gordon**, Director, Immunology, **Ask Bio**

# CONFERENCE DAY ONE - WEDNESDAY, OCTOBER 19

8AM - 5PM

12.30 **Immunogenicity Profile in Wild-Type Ferrets After Treatment With SP-101 & Doxorubicin: Inhalation Delivery of AAV Gene Therapy for the Treatment of Cystic Fibrosis**

- Sharing the impact of inhalation delivery on the post-dose capsid neutralizing antibody (cell-based assay) profile
- Implementation of a post-dose capsid total antibody (IgG) response and the decision-making process leading to a screen and titer strategy
- Evaluation of ferret T-cell responses to the capsid and transgene measured via ELISPOT, the implications of measuring no or low responses

**Lillian Falese**, Associate Director, Bioanalytical Development, **Spirovant Sciences**

12.30 **Biosimulation Model-informed Gene Therapy Development: Predicting Human Dose and Immunogenicity**

- Regulatory-ready IG simulator developed in consortium with eight pharmaceutical companies, now licensed by FDA
- Applied to gene therapy for predicting human dose from pre-clinical data, to translational phases, and through clinical development
- Case studies highlighting application to various modalities: AAV, mRNA and gene editing

**Piet van der Graaf**, Senior Vice President, Head of Quantitative Systems Pharmacology, **Certara**

1.00 **Lunch Break & Networking**

## PRE-CLINICAL TRACK

### Better Understanding Cellular Immunity in a Pre-Clinical Setting

2.30 **Innate Immune Signalling Pathways Leading to Cellular Immune Responses as a Function of Target Organ & AAV Vector Dose**

- Understanding innate immune signalling pathways that tigger CD8+ T cell activation
- What is the role of specific antigen presenting cells in orchestrating the response?
- Examining the role of target organ and vector dose in the response

**Roland Herzog**, Director, Gene & Cell Therapy Program, **Indiana University**

3.00 **Overcoming Innate Immune Barriers that can Impede AAV Gene Therapy Vectors**

- Overview of innate immune responses in AAV-based gene therapy
- Discuss the role of complement system in AAV immunogenicity
- Examining potential immunomodulatory strategies to circumvent AAV related immune responses

**Anna Majowicz**, Preclinical Immunology Lead, **Spark Therapeutics**

## CLINICAL TRACK

### Better Understanding Innate Immune Responses in the Clinical Setting

2.30 **Optimizing AAV Clinical Safety: Evolving Immunomodulation in the Phase 1 Study of RP-A501 (AAV9.LAMP2B) in Danon Disease**

- Overview of immunologic side effects associated with systemic AAV therapy
- Review of the safety and preliminary efficacy in the adult and pediatric cohorts of the Phase 1 Danon disease study
- Overview of complement activation and enhancements in the immunomodulatory regimen for the pediatric cohort

**Jonathan Schwartz**, Chief Scientific Officer, **Rocket Pharma**

3.00 **Roundtable Discussion: Discussing the Role of Innate Immunity in Clinical Manifestations of Immunogenicity**

- Immunogenicity of AAV is complex and challenging, involving elements of innate, humoral and cellular immunity that can adversely impact patient eligibility, safety, efficacy, durability of treatment, and ability to re-dose. Innate immunity is widely accepted to be the first in a chain of immune responses but there is still much to be understood. This roundtable will set out to discuss: current understandings of Innate immune responses to the Capsid & Transgene & its impact on long term & short-term efficacy & safety?

3.30 **Afternoon Refreshments & Networking**

## BETTER UNDERSTANDING THE IMMUNE RESPONSE TO GENE THERAPY & GENE EDITING PRODUCTS

4.00 **Anti-Cas9 Immunity & AAV-CRISPR Gene Editing in the Liver**

- Pre-existing immunity to Cas9
- Understand the consequences of anti-Cas9 immune responses for AAV-CRISPR in the liver
- Strategies to preserve gene edited hepatocytes

**William Lagor**
Associate Professor, Molecular & Cellular Biology
**Baylor College Medicine**

4.30 **Immune Response to Long-Term CAS9 Expression in Mice & Nhps in the Context of Crispr-Gndm® Mediated Gene Modulation**

- CRISPR/Cas9-mediated in-vivo gene editing offers great potential to treat several genetic disorders
- Conventional gene editing mediated through DNA strand breaks leads to off-target gene changes and sometimes cause fatal consequences
- Our proprietary CRISPR-GNDM® (Guide Nucleotide-Mediated Modulation) platform (aka CRISPRa/i), modulates gene expression without DNA strand breaks has great potential to overcome this hurdle

**Rajakumar Mandraju**
Principal Scientist
**Modalis Therapeutics**

5.00 **Engineered Treg Therapy for Tolerance to Anti-Drug Antibody Development**

- Introduction of Tregs and their importance in protein and gene replacement therapy with a few examples
- Overview of different engineered receptor designs
- Engineered Tregs for ADA development, using FVIII as an example. Will present published and unpublished information in the

**Moanaro Biswas**
Assistant Professor, Research
**Indianna University**

5.30 **Chairs Closing Remarks & End of Day 1**

# CONFERENCE DAY TWO - THURSDAY, OCTOBER 20

8AM - 5PM

8.50 **Chair's Opening Remarks**

## A DEEP DIVE INTO IMMUNOGENICITY ASSAY DEVELOPMENT CHALLENGES

9.00 **Clinical Enrolment Assay to Detect Pre-existing Neutralizing Antibodies to AAV6 With Demonstrated Transgene Expression in Gene Therapy Trials**

- Clinical enrollment cut off determination for pre-screening patients supporting AAV6 gene therapy trials
- Does assay format matter? NAb or TAb approach for clinical trial eligibility criteria
- Explore AAV6 seroprevalence evaluations

**Liching Cao**
Director, Bioanalytical Science
**Sangamo Therapeutics**

9.30 **Employing AAV Immunogenicity Predictive Models for Risk Assessment of Gene Therapy Products**

- Learn about the use of novel assays and platforms that can be implemented during the developability of gene therapy products
- Develop and implement novel predictive immune assays for product characterization and risk assessment of critical quality attributes (CQA) testing
- Understand the mechanism related to AAV-mediated immune responses which can provide potential solutions to modulate vector immunogenicity

**Inderpal Singh**
Immunogenicity Prediction Lead
**Spark Therapeutics**

10.00 **Morning Refreshments**

### PRE-CLINICAL TRACK

#### Pre-clinical Evaluation of Immunogenicity and Transduction

11.30 **Reduction of Peripheral Transduction of AAV9 Delivered to the CNS**

- IVIG pre-treatment block peripheral organ transduction of AAV9 delivered to the CNS
- IVIG pre-treatment reduces humoral response to AAV9 vector
- AAV9 transduces DRGs (Dorsal Root Ganglion) directly from the CNS compartment

**Roberto Calcedo**, VP, Preclinical & Immunology, **Affinia Therapeutics**

12.00 **Approach for Modeling and Species-Translation of Biodistribution, Transduction and Efficacy of AAV-based Gene Therapies**

- Data-driven identification of Dose, Serotype, Construct, Target tissue and Species dependencies of AAV treatment response
- Role of modeling in preclinical evaluation of AAV gene therapies - optimizing biodistribution study designs
- Use QSP modeling frameworks for species translation and minimum effective dose determination

**Jatin Narula**, Principal Scientist, BioMedicine Design, **Pfizer**

### CLINICAL TRACK

#### Immunogenicity Responses to Alternate Routes of Administration

11.30 **The Effect of Administration Route on Immune Response & How Viable Alternative Routes of Administration may be to Circumvent Immunogenicity**

- Assessing the impact of pre-existing immunity on effective intramoral AAV administration

**Ashlyn Bassiri**, VP, Immunology, **Kriya Therapeutics**

12.00 **Modulating AAV-Mediated Immune Response Depending on Route of Administration & Underlying Disease**

- Consideration for prophylactic immunomodulation/ immunosuppression depending on AAV-based gene therapy route of administration
- Finding a subtle balance between safe immunomodulation and sufficient suppression of AAV-driven immune response
- Additional safety considerations as they relate to immunosuppression when treating frail patient populations

**Olga Uspenskaya**, VP, Clinical Development, **Prevail Therapeutics**

12.30 **Lunk & Networking**

# CONFERENCE DAY TWO - THURSDAY, OCTOBER 20

8AM - 5PM

| PRE-CLINICAL TRACK | CLINICAL TRACK |
|---|---|
| **Exploring Efforts to Evade or Reduce the Immune Response** | **Immunogenicity Monitoring in a Clinical Setting** |
| 2.00 **Circumventing Immunogenicity in Gene Therapy With Svec, a Novel SV40 Based Viral Vector Platform**<br>· The crucial role of immunity and tolerance in gene therapy<br>· Features and development of the SVec platform in Haemophilia<br>· Opening new frontiers in gene therapy: Potential to restore tolerance in autoimmunity<br>**Steen Klysner**, Chief Executive Officer, **Amarna Therapeutics** | 2.00 **Clinical Immunogenicity Assessment Strategies for Cell & Gene Therapy**<br>· Discussing LNP based gene therapies<br>· Examining Allogenic CART therapies in the context of clinical assessment strategies and cross learning opportunities<br>· Clonality in allogenic CART therapies<br>**Priya Chockalingam,** VP, Translational Research, **Beam Therapeutics** |
| 2.30 ***IN VITRO* Immunogenicity Assessment of AAV Gene Therapy Vectors**<br>· Selecting less inflammatory AAV vector candidates<br>· Identifying immunogenic epitopes in AAV capsids<br>· Setting up the tools for immunosafety assessment<br>**Rebecca Xicluna**, Post Doctoral Fellow, **Roche** | 2.30 **Applying an Immunogenicity Risk-Based Approach to Gene Therapy Products**<br>· Current regulatory expectations<br>· Immune responses to gene therapies<br>· Evaluation and mitigation strategies<br>**Laura Salazar-Fontana, Independent Consultant** |

3.00 **Afternoon Refreshments & Networking**

## UNDERSTANDING THE REALITY & LIKELIHOOD OF RE-DOSING

3.30 **Understanding & Engineering Immune Responses for Better Gene Therapies**

· Review of current state of immune suppression and immune responses to gene therapy, particularly adaptive immune responses
· Novel immune modulatory strategies
· Development of engineered T regulatory cells for mitigating immune responses to gene therapy

**Allison Keeler**
Assistant Professor
**Horae Gene Therapy Center**

4.00 **Roundtable Discussion: If Re-Dosing Is a Reality, Then What Must The Field Be Prepared to Face?**

The immune response to systemic use of gene therapy is challenge that looms over the field, particularly as questions regarding durability of gene therapy products means that re-administration seems to look like an increasingly likely reality for many diseases targeted by this drug.

Re-dosing or re-administration is limited by a unique set of challenges itself. The biggest of which being the formation of an anti-AAV and anti-transgene immune response. Consequently, in cases where re-dosing is desirable or unavoidable, immunogenicity stands to be the biggest challenge that the field must work to overcome.

As such this roundtable discussion will set out to address:
· Durability of gene therapy products and its implications for re-dosing
· Immune modulation and immunosuppression strategies as an approach to dampen and mitigate immune response
· If re-dosing is a reality, then what must we be prepared to face?
· What effect does repeat exposure have on the immune response?
· The challenges of patient safety in re-dosing
· What can we expect the need and feasibility of re-dosing to be in the next 10 years?

5.00 **Chair's Closing Remarks**

5.10 **End of Conference**

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| Conference Only | $2,699 (Save $300) | $2,999 |
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| Conference Only | $2,299 (Save $300) | $2,599 |
| **Standard Pricing** | **Register & Pay By Friday, September 23** | **On The Door Rate** |
| Conference + Workshop Day | $4,596 (Save $300) | $4,898 |
| Conference Only | $3,699 (Save $300) | $3,999 |

All prices shown in USD. ** Academics and Not for Profit Organizations are eligible to obtain the Discounted Rate Pricing. Please contact info@hansonwade.com if you require more information. A special rate of $250 is available for Patient Advocacy and Charity Organizations.

Please visit the website for more information on pricing.

## Multi-Pass Discounts

**10% discount**
3 passes

**15% discount**
4 passes

**20% discount**
5+ passes

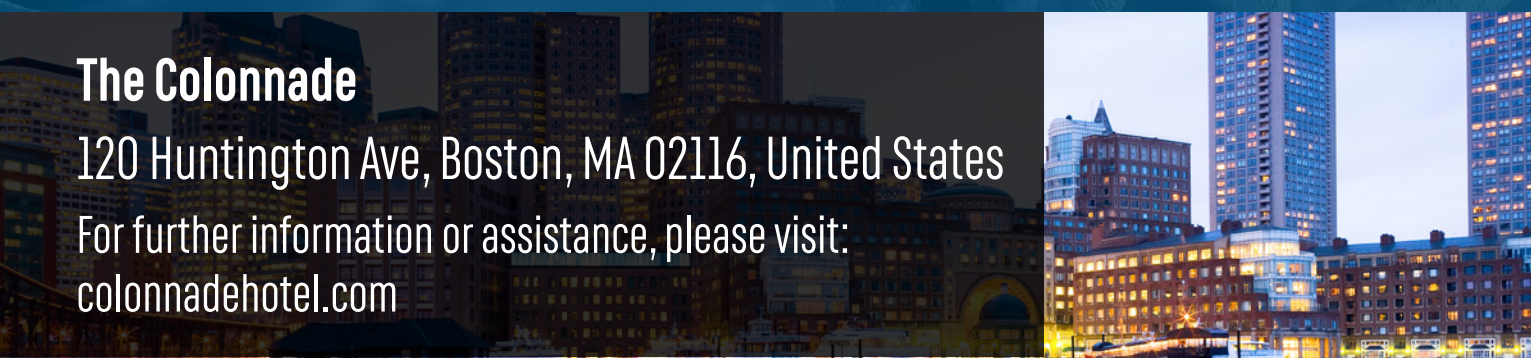
Please note that discounts are only valid when three or more delegates from one company book and pay at the same time. Discounts cannot be used in conjunction with any other offer or discount. Only one discount offer may be applied to the current pricing rate. Contact: register@hansonwade.com

## 3 Easy Ways To Book

genetherapy-immunogenicity.com

register@hansonwade.com

+1 617 455 4188



## The Colonnade

120 Huntington Ave, Boston, MA 02116, United States

For further information or assistance, please visit:
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