BOOK BY FRIDAY, SEPTEMBER 13 & SAVE UP TO $900

December 10-12, 2019
Boston, MA, USA

GTxN

Gene Therapy for Neurological Disorders

Overcome the Translational Challenges of Developing Efficacious Gene Therapies Targeting Neurological Disorders

19 Expert Speakers, Including:

Gregory LaRosa
Senior Vice President & Chief Scientific Officer, Rare Disease Research Unit
Pfizer

Lamya Shihabuddin
Head of Genetic Neurologic Diseases Research Cluster
Sanofi

Omar Khwaja
Chief Medical Officer
Voyager

Mark Milton
Area Head of Ophthalmology Therapeutic & PK Sciences
Novartis

"Expertly curated content"
M. Scott Bowers, Associate Director of Translational Science, **Aptinyx**

Tel: +1 617 455 4188 **Mail:** info@hansonwade.com www.gtxn-summit.com

hansonwade

The Gene Therapy for Neurological Disorders Summit (GTxN)

Discuss Novel Gene Therapy Delivery Routes to Effectively Target the CNS, Optimize Preclinical Models to Confidently Translate into the Clinic & Mitigate Immunogenicity to Deliver an Efficacious Dose

Built with the field's thought-leaders from the likes of **Pfizer**, **Biogen**, **Voyager**, **Novartis**, **GTxN** is the industry's first and only meeting dedicated to solving your translational drug development challenges, enabling you to accelerate the development of your neurological gene therapy candidate.

Attend GTxN to:

- Review recent gene therapy clinical successes and failures targeting neurological disorders to **identify improvement areas and opportunities to guide your future research**
- Discover the latest advances in administration routes and vector engineering to **improve delivery success**
- Address immunogenicity and the dosage challenges seen with gene therapies to **identify the optimal therapeutic window**

Join 60+ of your peers at this definitive conference over 3 days, 2 interactive workshops, 6+ hours of dedicated networking time and 19 expert industry speakers. Network with other leading minds in CNS gene therapy to form connections for future partnerships

What other attendees say about the World CNS Series:

"Outstanding conference"
Richard Wyse, Director of R&D, **The Cure Parkinson's Trust**

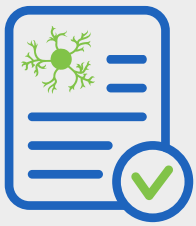
"Excellent networking and learning opportunity for pharma scientists"
Matthew Kennedy, Director, **Merck**

"Excellent conference with a very good and diverse set of speakers and topics"
Jim Cassella, Chief Development Officer, **Concert Pharmaceuticals**

At GTxN you will:

Discover novel gene therapy delivery advances to **effectively target the CNS**

Optimize your preclinical models to **confidently translate from *in vitro* to *in vivo***



Align your preclinical research with current regulation and clinical considerations **to improve the efficiency of your research**

Improve transduction efficiency and exploring advances in technology for monitoring gene expression **to measure candidate success**

Discuss the future of CRISPR and gene editing for neurological disorders to **assess different genetic therapeutic approaches**

"Impressive line-up of industry speakers"
Sean Smith, Executive Director, **Merck**

Tel: +1 617 455 4188 **Mail:** info@hansonwade.com
www.gtxn-summit.com

hansonwade

Your 19 Expert Speakers

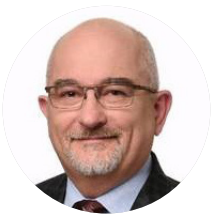
Boris Gorovits
Senior Director
Pfizer

Carl Morris
Chief Scientific Officer
Solid Biosciences

David Schaffer
Professor of Chemical & Biomolecular Engineering
UC Berkeley
Co-Founder & Acting Chief Scientific Officer
4D Molecular Therapeutics

David Theron
Prospective & Opportunities Director, CNS
Servier

Gabriele Proetzel
Director, Neuroscience External Research
Takeda



Gavin Corcoran
Chief Research & Development Officer
Axovant

Greg LaRosa
Senior Vice President & Chief Scientific Officer, Rare Disease Research Unit
Pfizer

Joyce Lo
Scientist II
Biogen

Klaudia Kuranda
Immunology Leader
Spark Therapeutics

Krzysztof Bankiewicz
Professor & Chair
University of California

Lamya Shihabuddin
Head, Genetic Neurologic Diseases
Sanofi

Mark Milton
Global Head, Pharmacokinetic Sciences Ophthalmology Therapeutic Area & Department Lead for Gene Therapies
Novartis

Martin Childers
Chief Medical Officer
AskBio

Martin Citron
Vice President, Neuroscience Research
UCB Pharma

Natasha Penner
Director, Clinical Pharmacology & Pharmacometrics
Biogen

Omar Khwaja
Chief Medical Officer & Head of Research & Development
Voyager Therapeutics

Robert Bell
Associate Research Fellow
Pfizer

Sander van Deventer
Executive Vice President, Research & Product Development
UniQure

Timothy MacLachlan
Executive Director, Global Head of Biologics Safety Assessment
Novartis

"A great opportunity to meet like-minded individuals and discuss the common issues that we all face in drug discovery. Really competent speakers and a great overall atmosphere"
Patrick Downey, Director, **UCB**

Pre-Conference Workshops
Tuesday, December 10

Workshop A
9:00am – 12:00pm

Measuring Gene Therapy Success in the CNS

Monitoring the delivery success of gene therapies in the CNS can be challenging. The imaging techniques and gene expression analysis advances will be discussed.

By attending this workshop, you will:

- Discuss gene therapy administration routes
- Discover innovative imaging techniques for improving target engagement quantification
- Evaluate current gene expression analysis techniques and the need for improvement in technology

Workshop Leader

Krystof Bankiewicz
Professor in Residence of Neurological Surgery & Neurology
University of California at San Francisco

Workshop B
12:30pm – 3:30pm

Discovering Capsid Engineering Technology Advances

Delivery is the greatest challenge for gene therapies targeting the CNS. Innovative vector technology is being developed to help improve the target engagement and cell specificity of gene therapy delivery.

Attend this workshop to:

- Explore the use of vector engineering to improve gene therapy delivery
- Discuss the promotors and regulators required for cell-specific expression
- Debate the use of non-viral vs viral vectors

Workshop Leader

David Schaffer
Professor of Chemical & Bimolecular Engineering & Director of the Berkeley Stem Cell Center
UC Berkeley
Co-Founder and Acting CSO
4D Molecular Therapeutics

> "Very focused meeting, that accomplished a lot in two days. The presentations were high quality and organized into interesting sessions"
>
> Sally Ishizaka, Senior Director, **Eisai**

Conference Day One
Wednesday, December 11

Omar Khwaja
Chief Medical Officer & Head of Research & Development
Voyager Therapeutics

8.30 **Chair's Opening Remarks**

Gene Therapy: The Promising Future for Neurological Disorders

Natasha Penner
Director, Clinical Pharmacology & Pharmacometrics
Biogen

8.45 **Beyond Small Molecules & Protein Therapeutics: New Modalities for Treatment of CNS Diseases**

- Evaluating the opportunities and limitations of new modalities in CNS drug development
- Targeting transcriptome with oligonucleotides and genome with gene therapies was shown effective in treating previously untreatable diseases such as spinal muscular dystrophy
- With oligonucleotides unable to penetrate the blood-brain barrier, assessing intrathecal administration directly into CSF to allow targeting all CNS cell types and pursue oligonucleotides for wide variety of neurodegenerative diseases
- Discussing the importance of distribution for achieving a desired effect

9.15 **Speed Networking**

This session is the ideal opportunity to meet face-to-face with the key thought leaders working in the neurological gene therapy field. Specifically designed to connect you with new contacts from the most active companies in the field, the renowned Speed Networking session will be one of the most valuable hours you spend at **GTxN**.

10.00 **Morning Refreshments**

Delivery, Delivery, Delivery: Accessing the CNS

Robert Bell
Associate Research Fellow
Pfizer

11.00 **Evaluating the Barriers to AAV Delivery into the Central Nervous System**

- Providing an overview of blood-brain barrier structure and function, brain ventricles and cerebral spinal fluid flow
- Assessing advantages and limitations with current AAV technology: routes of administration, dosage requirements, age considerations and immune responses
- Discussing the important considerations when evaluating new BBB technologies for drug delivery into the CNS

Omar Khwaja
Chief Medical Officer & Head of Research & Development
Voyager Therapeutics

11.30 **Achieving Therapeutic AAV-based Gene Delivery to the Brain & Nervous System**

- An overview of the challenges of gene delivery to the central and peripheral nervous system
- Review of key methods and challenges with application to the clinic
- Future advances in gene delivery

12.00 **Lunch & Networking**

Improving Specific Cell Targeting for More Efficient Cell Transduction

Martin Childers
Chief Medical Officer
AskBio

1.30 Impact of Capsid Engineering & Synthetic Components to Advance CNS Gene Therapy

- Hitting the bullseye with chimeric capsids
- Throwing a doggy-bone to improve DNA plasmids
- Opening new pathways with inducible promoters
- Increasing yields, enhancing potency, and reducing cost

Lamya Shihabuddin
Head, Genetic Neurologic Diseases
Sanofi

2.00 Optimizing Targeting & Gene Expression: Defining Disease, Region & Cell Type

- Developing gene therapies for various genetic neurologic disorders and considering route of administration and optimized targeting, both regional and cell type
- Discussing capsid selection and capsid engineering for next generation gene therapies
- Considering the long term effect and safety of gene therapy doses for neurological disorders

2.30 Afternoon Refreshments & Networking

Improving the Efficiency of Gene Therapy Development

David Theron
Prospective & Opportunities Director, CNS
Servier

3.00 Aligning Preclinical Research with Current Regulation & Clinical Considerations

- FDA and EMA have recently (2018-19) released guidance for development of gene therapies and rare diseases
- Adjusting research strategies to fit with new guidance
- Assessing the challenges associated with meeting current regulation whilst carrying out research relevant for translation to the clinic

Gavin Corcoran
Chief Research & Development Officer
Axovant

3.30 Achieving Clinical Progress while Establishing Mutually Beneficial Links with Academia

- Sharing case studies from the clinical development of AAV-based gene therapies for the treatment of GM1 and GM2 gangliosidosis
- Exploring strategies to interact with academic institutions in a more standardized and effective way
- Looking ahead: what's on the horizon for gene therapies and how will this future necessitate change in the field

Omar Khwaja
Chief Medical Officer & Head of Research & Development
Voyager Therapeutics

4.00 Chair's Closing Remarks

4.15 Scientific Poster Session

The learning and networking continues at the Poster Session, an informal part of the conference agenda, allowing you to connect with your peers in a relaxed atmosphere and continue to forge new, beneficial relationships. You will have the opportunity to present and review presentations displaying new data from preclinical gene therapy research and clinical gene therapy progress in neurological disorders.

Conference Day Two
Thursday, December 12

Omar Khwaja
Chief Medical Officer & Head of Research & Development
Voyager Therapeutics

8.30 Chair's Opening Remarks

Optimizing Transduction Efficiency & Measuring Target Engagement

Carl Morris
Chief Scientific Officer
Solid Biosciences

8.45 Translating Preclinical Responses: Addressing the Challenges of Gene Therapy

- Understanding the dose selection challenges
- Developing methods to measure effectiveness of treatment
- Investigating surrogate and non-invasive biomarkers for target engagement and efficacy

Joyce Lo
Scientist II
Biogen

9.15 Developing a CRISPR Tool to Facilitate Quantification of AAV Transduction in the Mouse CNS

- Several novel AAVs have been developed to achieve wide-scale transduction across multiple cells types in the central nervous system (CNS)
- Wide scale CNS gene transfer is relevant to gene therapy as well as for disease modeling in mice
- Developing a CRISPR-based method that enables analysis of AAV transduction in neuronal population in CNS in a more sensitive, quantitative and higher throughput manner
- Expanding the capacity of this method to allow analysis of AAV transduction in astrocytes and oligodendrocytes in the mouse CNS

Sander van Deventer
Executive Vice President, Research & Product Development
UniQure

9.45 Novel AAV-delivered Gene Silencing Technologies Targeting the CNS: Imaging of miRNAs & Therapeutic Efficacy in Huntington's Disease & SCA3

- Novel miRNA-based silencing devoid of off-target effects
- Spread of silencing through exosome-mediated secondary distribution of therapeutic miRNAs
- Efficacy in preclinical models of HD and SCA3

10.15 Morning Refreshments & Networking

Improving Preclinical Models for Calculating Dosage & Testing Toxicity

Martin Citron
Vice President, Neuroscience Research
UCB Pharma

10.45 Evaluating *In Vivo* Pharmacology Studies for Neurological Gene Therapies

- Reviewing how the role of *in vivo* pharmacology in neurologic disease drug development has changed
- Discussing the new demands for *in vivo* modeling that have been created by moving from symptomatic to disease modifying treatment, for the example of Parkinson's disease
- Exploring the additional layer of complexity for gene therapy, introduced by the species barrier, i.e. the different potency of AAV in mice vs. humans
- Developing a realistic assessment of these challenges, important in the development of neurological gene therapies

11.15 Mastermind Discussion: Discussing Current Preclinical Animal Models for Neurological Disorders

This mastermind session will host interactive discussions amongst your tables and allow you to share your opinions with your peers on the value of using in vivo models for preclinical gene therapy research.

What value do rodent studies provide?

Should large mammal disease models be more readily available?

Do animal models make any sense at all in this field except for safety studies?

Are organoid models better? Are patient-derived neurons better?

12.00 Lunch & Networking

Mitigating Immunogenicity & Dosage Challenges for Optimal Efficacy

Klaudia Kuranda
Immunology Leader
Spark Therapeutics

1.00 Immunogenicity of AAV - Implications for CNS Gene Transfer

- Reviewing innate and adaptive immune responses to AAV in human
- Exploring natural pre-existing immunity to AAV and how we are dealing with it
- Determining what we have learnt from clinical trials so far – failure of animal models to predict immunogenicity
- Discovering how to use peripheral blood to monitor immune response in CNS

Mark Milton
Global Head, Pharmacokinetic Sciences Ophthalmology Therapeutic Area & Department Lead for Gene Therapies
Novartis

1.30 Finding the Right Patient: The Balance Between Immunogenicity, Safety & Efficacy

- Addressing immunogenicity for neurological gene therapies
- Discussing the options for addressing pre-existing immunity
- Exploring the challenges of redosing with gene therapies

2.00 The Immunosuppression Debate

- How do we need to address immunogenicity for neurological disorders?
- Is immunosuppression a safe and viable option?
- Considering pre-existing immunity and re-dosing, how can we navigate the risks and rewards?

Mark Milton
Global Head, Pharmacokinetic Sciences Ophthalmology Therapeutic Area & Department Lead for Gene Therapies
Novartis

Carl Morris
Chief Scientific Officer
Solid Biosciences

Boris Gorovits
Senior Director
Pfizer

2.45 Afternoon Networking & Refreshments

Safeguarding the Future of Gene Therapy Patients

3.15 Panel Discussion: Discussing the Unknown Long Term Safety Issues of Gene Therapies

- Considering the success seen with neurological gene therapies and the potential for the future, are the current long-term safety regulations correct?
- Where can improvement be made on gene therapy safety precautions?
- What are the major risk factors to be considering for the gene therapy patients?

Gabriele Proetzel
Director, Neuroscience External Research
Takeda

Gavin Corcoran
Chief Research & Development Officer
Axovant

Greg LaRosa
Senior Vice President & Chief Scientific Officer, Rare Disease Research Unit
Pfizer

Timothy MacLachlan
Executive Director, Global Head of Biologics Safety Assessment
Novartis

Omar Khwaja
Chief Medical Officer & Head of Research & Development
Voyager Therapeutics

4.00 Chair's Closing Remarks

Partnership Opportunities

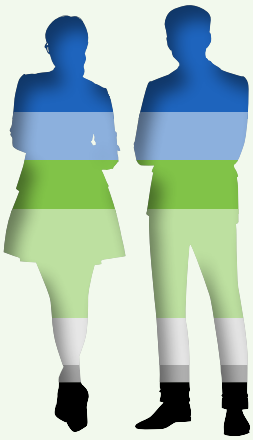
GTxN is the only industry-focused meeting dedicated to helping drug developers successfully develop gene therapies for neurological indications.

Network with leading minds from the likes of **Takeda**, **Servier**, **Axovant**, **UCB** and **Sanofi** to demonstrate how your products and services can help them to overcome their translational challenges and accelerate their gene therapy candidate to market.

As one of the few selected service providers at **GTxN**, we will work with you to build a bespoke partnership package to ensure you meet your 2020 business needs.

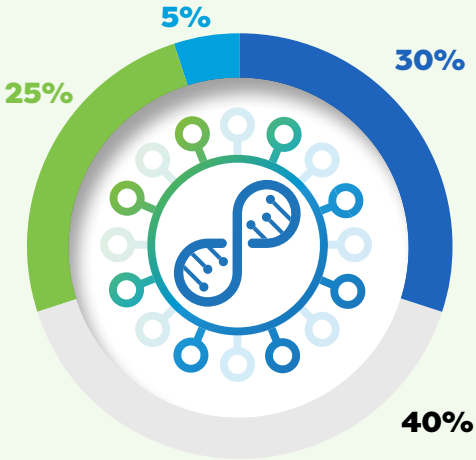
Get in touch for more information on how you can be involved at **GTxN** by emailing: **sponsor@hansonwade.com**

AUDIENCE SENIORITY



- Chief/CxO: **20%**
- President/VP: **10%**
- Global Head/Head Of: **7%**
- Director/Associate Director: **36%**
- Scientist: **11%**
- Professor: **2%**
- Other: **13%**

AUDIENCE COMPANIES



> "Very well organized conference with exceptional speakers. Well represented across pharma, biotech, academics and service providers"
>
> Linda Hedley, Business Development Consultant, **SRI International**

Get Involved

Okeefe Ogholo
Business Development Manager
Tel: +1 617 455 4188
Email: sponsor@hansonwade.com

Ready to Register?

3 Easy Ways To Book

www.gtxn-summit.com

Tel: +1 617 455 4188

Email: register@hansonwade.com

1. Network with CNS gene therapy thought-leaders to **establish new and meaningful connections**
2. Widen your knowledge of the different gene therapies targeting neurological disorders being developed to **learn of their progress and benchmark yourselves against your competitors**
3. Share your research at the Poster Session to gain feedback from your peers and **create valuable collaborations with your peers to facilitate your research**

SECURE YOUR PLACE

Package Details	Book by Friday, September 13	Standard Price
GOLD Conference + 2 Workshops	$3097 **(save $900)**	$3697 **(save $300)**
SILVER Conference + 1 Workshop	$2698 **(save $700)**	$3198 **(save $200)**
BRONZE Conference Only	$2099 **(save $600)**	$2799
Workshops (Each)	$599	

*Special 40% off for academics available upon request

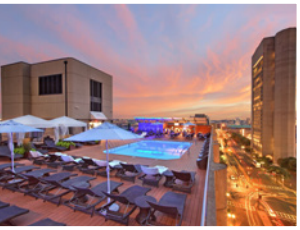
Team Discounts*

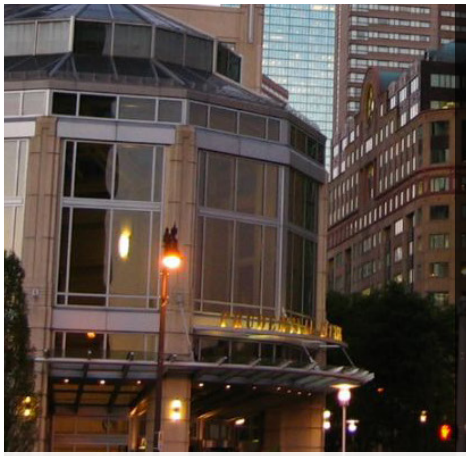
- **10% discount – 3 delegates**
- **15% discount – 4 delegates**
- **20% discount – 5 or more delegates**

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

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





VENUE

The Colonnade
120 Huntington Avenue, Boston

For further information or assistance, please visit www.colonnadehotel.com