GTxN
Gene Therapy for Neurological Disorders

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

27 Expert Speakers, Including:

- **Gregory LaRosa**
  Senior Vice President & Head of Scientific Research, Rare Disease Research Unit
  Pfizer

- **Lamya Shihabuddin**
  Head of Genetic Neurologic Diseases Research Cluster
  Sanofi

- **Omar Khwaja**
  Chief Medical Officer
  Voyager

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

Expertise Partner: PTC Therapeutics
Spotlight Partner: atuka
Panel Partners: Biocure, PPD Biotech, NeuExcell, Lonza
Exhibition Partners: Invicro
Event Partner: Hanson Wade

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

December 10-12, 2019
Boston, MA, USA

BOOK NOW & SAVE UP TO $300

Overview

- **December 10-12, 2019**
  Boston, MA, USA

- **GTxN**
  Gene Therapy for Neurological Disorders

- **27 Expert Speakers**
  Including:
  - Gregory LaRosa
    Senior Vice President & Head of Scientific Research, Rare Disease Research Unit
    Pfizer
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    Therapeutic Area & Department Lead for Gene Therapies
    Novartis

- **Panel Partners**
  - Biocure
  - PPD Biotech
  - NeuExcell
  - Lonza

- **Exhibition Partners**
  - Invicro

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

- **Overview of Event**
  - Overcome the Translational Challenges of Developing Efficacious Gene Therapies Targeting Neurological Disorders
  - December 10-12, 2019
  - Boston, MA, USA

- **Registration**
  - Book now and save up to $300

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 27 expert industry speakers. Network with other leading minds in CNS gene therapy to form connections for future partnerships

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

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

Impressive line-up of industry speakers
Sean Smith, Executive Director, Merck
Your 27 Expert Speakers

Boris Gorovits  
Senior Director  
Pfizer

Carl Morris  
Chief Scientific Officer  
Solid Biosciences

Carol Satler, M.D., Ph.D.  
Vice President, Regional Medical Officer  
PPD Biotech

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

David Theron  
Early Clinical Development Director  
Servier

Eloise Hudry  
Senior Investigator, Cell & Gene Therapies  
Novartis

Emily Hickey  
Chief Operations Officer  
Biomere

Gabriele Proetzel  
Director, Neuroscience External Research  
Takeda

Gavin Corcoran  
Chief Research & Development Officer  
Axovant

Greg LaRosa  
Senior Vice President & Head of Scientific Research, Rare Disease Research Unit  
Pfizer

Ira Goodman  
Principal Investigator  
Compass Clinic

Jodi Cook  
Head of Gene Therapy Strategy  
PTC Therapeutics

Jonathan Brotchie  
Co-Founder, Director & President  
Atuka Inc

Joyce Lo  
Scientist II  
Biogen

Klaudia Kuranda  
Immunology Leader  
Spark Therapeutics

Lamya Shihabuddin  
Head, Genetic Neurologic Diseases  
Sanofi

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

Mark Nedelman  
Chief Executive Officer  
Biomere

Martin Childers  
Chief Medical Officer  
AskBio

Martin Citron  
Vice President & Head, Neuroscience TA  
UCB Pharma

Natasha Penner  
Director, Clinical Pharmacology & Pharmacometrics  
Biogen

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

Petra Kauffman  
Vice President, R&D Translational Medicine  
AveXis

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

Timothy Miller  
Vice President & Area Head, Pediatrics & Rare Diseases  
PPD

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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

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 promoters and regulators required for cell-specific expression
- Debate the use of non-viral vs viral vectors

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**

<table>
<thead>
<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speaker(s)</th>
<th>Notes</th>
</tr>
</thead>
<tbody>
<tr>
<td>8.15</td>
<td><strong>Chair’s Opening Remarks</strong></td>
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<tr>
<td>8.30</td>
<td><strong>Gene Therapy: The Promising Future for Neurological Disorders</strong></td>
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</tbody>
</table>
| 8.30  | **Beyond Small Molecules & Protein Therapeutics: New Modalities for Treatment of CNS Diseases** | Natasha Penner (Director, Clinical Pharmacology & Pharmacometrics, Biogen) | - 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 |
| 9.00  | **Targeted Micro-Dosing Strategy in Gene Therapy to Deliver Clinical Results** | Jodi Cook (Head of Gene Therapy Strategy, PTC Therapeutics)                | - Using targeted micro-doses for systemic gene therapy to allows for greater efficacy, durability, lower risk of immunogenicity or other off-target effect  
- Discussing data from PTC gene therapy clinical trials  
- Efficient and scalable manufacturing  
- Understanding the value behind one-time injections |
| 9.30  | **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. |
| 10.15 | **Morning Refreshments**                                                     |                                                                          |                                                                      |
| 10.45 | **Delivery, Delivery, Delivery: Accessing the CNS**                         |                                                                          |                                                                      |
| 10.45 | **Evaluating the Barriers to AAV Delivery into the Central Nervous System** | Robert Bell (Associate Research Fellow, Pfizer)                         | - 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 |
| 11.15 | **Achieving Therapeutic AAV-based Gene Delivery to the Brain & Nervous System** | Omar Khwaja (Chief Medical Officer & Head of Research & Development, Voyager Therapeutics) | - 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 |
| 11.45 | **Introduction to Atuka**                                                    | Jonathan Brotchie (Co-Founder, Director & President, Atuka Inc)           |                                                                      |
| 12.00 | **Lunch & Networking**                                                      |                                                                          |                                                                      |

**Gene Therapy for Neurological Disorders**
December 10-12, 2019 | Boston, MA, USA
Improving Specific Cell Targeting for More Efficient Cell Transduction

**0.00 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

**1.30 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

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Improving the Efficiency of Gene Therapy Development

**2.00 Panel Discussion: The Gene Therapy Journey in Neurologic Disease: Designing Clinical Programs to Meet Patient Needs**
- Understanding and balancing the burden of gene therapy clinical trials from the patient’s perspective
- What hurdles can we realistically lessen or eliminate for patients pursuing gene therapies – perspectives from the sponsor, CRO and investigator
- How to plan for a more patient-centric trial in early development

**Panelists:**
- **Carol Satler, M.D., Ph.D.**
  Vice President, Regional Medical Officer
  PPD Biotech
- **Ira Goodman**
  Principal Investigator
  Compass Clinic
- **Timothy Miller**
  Vice President & Area Head, Pediatrics & Rare Diseases
  PPD

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**2.30 Afternoon Refreshments & Networking**

**3.30 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

**4.00 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

**4.30 Chair’s Closing Remarks**

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**4.45 Drinks & 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

**8.30**  
Chair’s Opening Remarks  

**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

**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 cell 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

**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

### Optimizing Transduction Efficiency & Measuring Target Engagement

**Carl Morris**  
Chief Scientific Officer  
Solid Biosciences

**Joyce Lo**  
Scientist II  
Biogen

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

**Martin Citron**  
Vice President & Head, Neuroscience  
TA UCB Pharma

### Improving Preclinical Models for Calculating Dosage & Testing Toxicity

**11.15**  
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

**11.45**  
Panel Discussion: Discussing Current Preclinical Animal Models for Neurological Disorders  
- 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?

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

**Panelists:**  
Emily Hickey  
Chief Operations Officer  
Biomere  
Martin Citron  
Vice President & Head, Neuroscience  
TA UCB Pharma  
Mark Nedelman  
Chief Executive Officer  
Biomere
### 12.15 Lunch & Networking

**Mitigating Immunogenicity & Dosage Challenges for Optimal Efficacy**

<table>
<thead>
<tr>
<th><strong>1.15 Immunogenicity of AAV – Implications for CNS Gene Transfer</strong></th>
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<tbody>
<tr>
<td>Klaudia Kuranda  &lt;br&gt;Immunology Leader  &lt;br&gt;Spark Therapeutics</td>
</tr>
<tr>
<td>• Reviewing innate and adaptive immune responses to AAV in human  &lt;br&gt;• Exploring natural pre-existing immunity to AAV and how we are dealing with it  &lt;br&gt;• Determining what we have learnt from clinical trials so far – failure of animal models to predict immunogenicity  &lt;br&gt;• Discovering how to use peripheral blood to monitor immune response in CNS</td>
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<tr>
<th><strong>1.45 Finding the Right Patient: The Balance Between Immunogenicity, Safety &amp; Efficacy</strong></th>
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</thead>
<tbody>
<tr>
<td>Mark Milton  &lt;br&gt;Global Head, Pharmacokinetic Sciences  &lt;br&gt;Ophthalmology Therapeutic Area &amp; Department Lead for Gene Therapies  &lt;br&gt;Novartis</td>
</tr>
<tr>
<td>• Addressing immunogenicity for neurological gene therapies  &lt;br&gt;• Discussing the options for addressing pre-existing immunity  &lt;br&gt;• Exploring the challenges of redosing with gene therapies</td>
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</table>

#### 2.15 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?

**Panelists:**

- **Mark Milton**  <br>Global Head, Pharmacokinetic Sciences  <br>Ophthalmology Therapeutic Area & Department Lead for Gene Therapies  <br>Novartis
- **Carl Morris**  <br>Chief Scientific Officer  <br>Solid Biosciences
- **Boris Gorovits**  <br>Senior Director  <br>Pfizer

### 2.45 Afternoon Networking & Refreshments

**Safeguarding the Future of Gene Therapy Patients**

<table>
<thead>
<tr>
<th><strong>3.15 Trial Design Considerations for Rare Diseases</strong></th>
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<tbody>
<tr>
<td>Petra Kauffman  &lt;br&gt;Vice President, R&amp;D Translational Medicine  &lt;br&gt;AveXis</td>
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<tr>
<td>• Discussing the use of natural history datasets  &lt;br&gt;• Addressing the need for suitable endpoints  &lt;br&gt;• Exploring patient-focused approaches</td>
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<thead>
<tr>
<th><strong>3.45 Panel Discussion: Discussing the Unknown Long Term Safety Issues of Gene Therapies</strong></th>
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<tbody>
<tr>
<td>Gabriele Proetzel  &lt;br&gt;Director, Neuroscience External Research  &lt;br&gt;Takeda</td>
</tr>
<tr>
<td>Gavin Corcoran  &lt;br&gt;Chief Research &amp; Development Officer  &lt;br&gt;Axovant</td>
</tr>
<tr>
<td>Greg LaRosa  &lt;br&gt;Senior Vice President &amp; Head of Scientific Research, Rare Disease Research Unit  &lt;br&gt;Pfizer</td>
</tr>
<tr>
<td>Timothy MacLachlan  &lt;br&gt;Executive Director, Global Head of Biologics Safety Assessment  &lt;br&gt;Novartis</td>
</tr>
</tbody>
</table>

- 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?

### 4.30 Chair’s Closing Remarks

**Omar Khwaja**  <br>Chief Medical Officer & Head of Research & Development  <br>Voyager Therapeutics
**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

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**A U D I E N C E  S E N I O R I T Y**

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

**A U D I E N C E  C O M P A N I E S**

- Large Pharmaceutical: 30%
- Small Biotech: 40%
- Service Providers: 25%
- Academia: 5%

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**Get Involved**

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**GTxN Partners**

**Expertise Partner**

PTC Therapeutics is an industry leader in the discovery, development and commercialization of medicines for the treatment of devastating rare disorders. For over 20 years, PTC has been pursuing innovative technologies and therapies to develop and provide access to clinically-differentiated medicines, including gene therapies. PTC believes that by using proven scientific technologies, they can find innovative ways to treat rare disorders to create more shared moments and opportunities for patients and their families.

www.ptcbio.com

**Spotlight Partner**

Atuka provides contract research and consultancy services for the biopharmaceutical industry with world-leading expertise in Parkinson’s disease and related neurological conditions. We provide cutting-edge, rodent and non-human primate models (toxin and molecular pathology-driven) to evaluate efficacy and target engagement over a comprehensive range of symptomatic, motor (e.g. parkinsonism and dyskinesia), non-motor (e.g. cognition and impulse control) and disease-modification assays. Atuka also offers medicinal chemistry, DMPK and in-vivo imaging services to aid development of novel therapeutics.

www.atuka.com

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**Okeefe Ogholo**

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

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www.gtxn-summit.com
Panel Partner

NeuExcell is an early stage gene therapy company focusing on neurodegenerative diseases. We have developed a disruptive neural repair technology through astrocyte-to-neuron conversion. Our vision is to improve the quality of life of millions of patients worldwide who are suffering from neurodegenerative conditions by using the power of gene therapy to restore damaged neural tissue.

www.neuexcell.com

Panel Partner

PPD Biotech is devoted to finding the right Phase I-IV clinical development solutions for biotech and small to midsize pharma. With dedicated teams, PPD Biotech combines the global resource scalability and full-service offerings of PPD with personal attention, flexibility and a shared mindset that enables us to find the optimal solutions for your study, no matter the size.

We bring a comprehensive set of expertise to gene therapy research – from product development and regulatory strategists, labs services and clinical execution through to pre- and post-approval work. We have the right science, trial designs and experienced operations teams to move your gene therapy studies forward.

www.ppdbiotech.com

Exhibition Partner

Lonza Pharma & Biotech

We provide contract development and manufacturing services that enable pharma and biotech companies to deliver medicines to patients. From the building blocks of life to the final drug product, our solutions are created to simplify your outsourcing experience and provide a reliable outcome when you expect it. Our extensive track record includes commercialization of pioneering therapies and manufacturing of a wide variety of therapies. Together, let’s bring the next medicine to life.


Event Partner

Invicro

Founded in 2008, Invicro’s mission is to improve the role and function of imaging in drug discovery across all therapeutic areas and phases. As part of the Konica Minolta Precision Medicine Initiative, we combine operational excellence with scientific, medical and analytic expertise to de-risk drug development from preclinical to late phase. The company’s innovative quantitative tissue biomarker services help uncover novel insights and support the development of more personalized therapies.

www.invicro.com

Get Involved

Okeefe Ogholo
Business Development Manager
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SECURE YOUR PLACE

<table>
<thead>
<tr>
<th>Package Details</th>
<th>Standard Price</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>GOLD</strong> Conference + 2 Workshops</td>
<td>$3,697 (save $300)</td>
</tr>
<tr>
<td><strong>SILVER</strong> Conference + 1 Workshop</td>
<td>$3,198 (save $200)</td>
</tr>
<tr>
<td><strong>BRONZE</strong> Conference Only</td>
<td>$2,799</td>
</tr>
<tr>
<td>Workshops (Each)</td>
<td>$599</td>
</tr>
</tbody>
</table>

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

*Special 40% off for academics available upon request.

VENUE

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