

INTERNATIONAL NEUROTRANSMITTER DISORDERS

# CONFERENCE

LONDON

5TH - 7TH NOVEMBER 2025

### PRELIMINARY AGENDA

BRIDGING SCIENTIFIC INNOVATION & CLINICAL ADVANCES FOR PATIENTS





# Medical & Scientific Organising Committee

### Professor Manju Kurian

Medical & Scientific Committee Co-Chair UCL Professor of Genetics | Honorary Consultant in Paediatric Neurology London, UK

### **Professor Simon Heales**

Medical & Scientific Committee Co-Chair UCL Professorial Fellow | Consultant Clinical Scientist London, UK

### Dr Simon Pope

Medical & Scientific Committee Co-Chair UCL Senior Research Fellow | Principle Biomedical Scientist London, UK

### Professor Thomas Opladen

Center for Pediatric and Adolescent
Medicine | Division of Pediatric Neurology
& Metabolic Medicine,
Heidelberg, Germany

### Dr Alpa Dherai

Consultant Biochemist Mumbai, India

### Professor Rafa Artuch

Head of Biochemistry Department Barcelona, Spain

### Dr Roser Pons

Pediatric Neurologist & Faculty Member Athens, Greece

### Dr Toni Pearson

Pediatric Neurologist & Movement Disorder Specialist | Lead Neurologist, Gene Therapy Trials, US St Louis, USA

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# "Bridging Scientific Innovation & Clinical Advances for Patients."

### **OUR PROMISE...**

As the INDConf 2025 Medical & Scientific Committee, we are committed to ensuring that every discovery moves us closer to better care, deeper understanding and real hope for families affected by neurotransmitter disorders!

### INDConf 2025 Venue

### Goodenough College - LONDON

Mecklenburgh Square, London, WC1N 2AB

Nestled in the heart of Bloomsbury, Goodenough College offers a truly special setting for the INDConf 2025. This historic LONDON venue blends academic charm with a warm, welcoming atmosphere - perfect for meaningful connection, learning and collaboration.

www.goodenoughhouse.co.uk







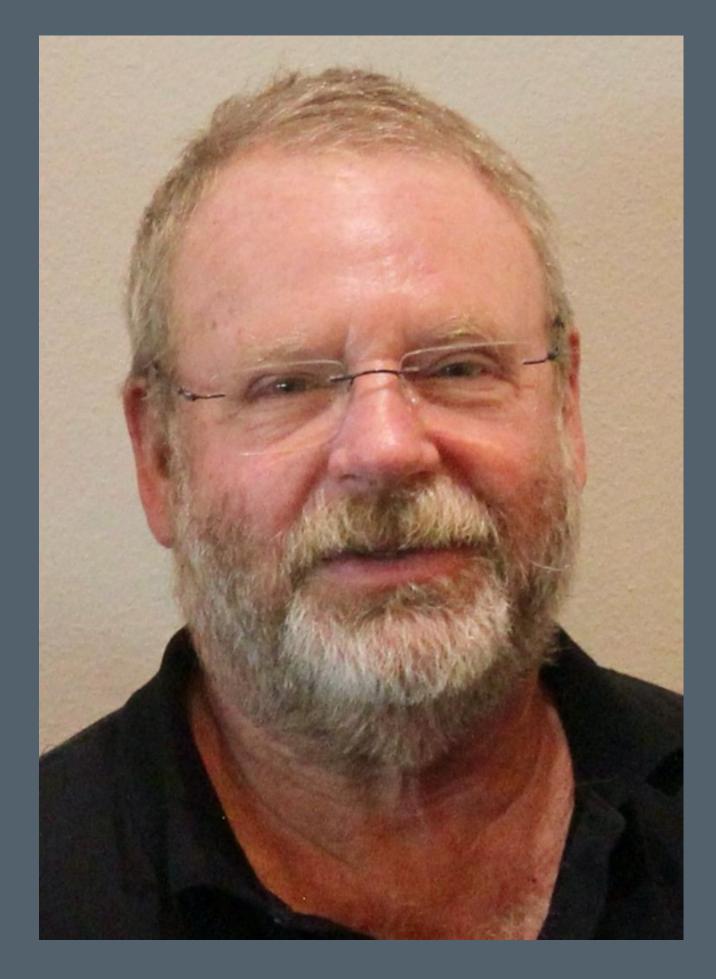




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Professor Keith Hyland

# Joint Keynote Lecture

Wednesday 5th November

We are honoured to welcome two of the fields most influential pioneers, Professor Keith Hyland and Professor Simon Heales, for a joint keynote reflecting on decades of discovery, collaboration, and impact in the diagnosis and understanding of neurotransmitter disorders. Their combined contributions have shaped the very foundations of neurotransmitter testing and care, inspiring generations of clinicians and researchers globally. Together, they offer a unique perspective on the past, present and future of this evolving field - and remind us of the power of science driven by dedication, curiosity and compassion.



Professor Simon Heales

# Day

### Wednesday 5th November

### Registration & Lunch

Join us from 12:00 PM for arrival registration and a light lunch. This is a great opportunity to settle in, collect your materials and connect with fellow delegates before the conference officially begins...

### Session I 13:30 - 15:00PM

An Overview of Neurotransmitter Disorders.

A brief introduction to the biology, cinical features and significance of rare neurotransmitter disorders, setting the stage for deeper exploration througout the conference

### Coffee & Poster Viewing 15:00 - 15:30PM

Enjoy coffee and explore poster presentions at your leisure

### Session 2 15:30 - 17:30PM

**Advances in Laboratory Diagnostics: Biochemical & Genetic Testing.** 

Discover the latest tools and techniques transforming biochemical and genetic testing for neurotransmitter disorders

### Welcome Drinks Reception 17:30 - 19:30PM

Join us for a welcome drinks reception with short talks on career journey and serendipitous connections in neurotransmitter disorders



# Day 2 Thursday 6th November

### Clinical Focus & Expert Exchange

Day two brings a packed programme of vital session, from in-depth clinical case discussion to the always-popular Meet the Experts - an unmissable day of insight and exchange.

Session 3 08:30 - 10:00AM

**Selected Oral Presentations.** 

An arrival coffee followed by a selection of 4 chosen oral presentations, each offering a 10-minute talk followed by a 5-minute discussion - highlighting emerging research and fresh perspectives across the field.

Session 4 10:00 - 12:45PM

Monoamine Neurotransmitter Disorders -Expanding Diseases and Phenotypes

Following a coffee break this session takes a closer look at the growing complexity of monoamine neurotransmitter disorders, with fresh insights into emerging conditions and their presentations.

Lunch & Poster Viewing 12:45 - 14:00PM

Enjoy lunch alongside a chance to view poster presentations and connect with fellow delegates.

Session 5 14:00 - 16:00PM

Vitamins, GABA and Glycine Disorders

This session delves into key disorders linked to vitamins, GABA and Glycine - shedding light on their genetic foundations, clinical impact and treatment approaches

Session 6 16:00 - 17:15PM

Disease Modelling for Monoamine Neurotransmitter Disorders

Grab a coffee before diving into cutting-edge disease modelling for monoamine neurotransmitter disorders, from in silico analysis to iPSC-base research.

Session 7 17:15 - 19:20PM

**Workshop for Clinical Cases & Video** 

We close the day with an interactive workshop featuring real clinical cases and video discussions an opportunity to reflect, learn and exchange insights in a practical setting. JOIN OUR WORKSHOP

# Clinical Case Discussion & Meet the EXPERTS

Bridging Scientific Innovation & Clinical Advances for Patients



# Day 3

### Friday 7th November

### Last Sessions, Presentations & Farewell Gathering

Welcome to our final day - packed with vital topics and lasting takeaways. Please do join us for lunch before departure at 1:45pm

Session 8 08:30 - 10:00AM

**Selected Oral Presentations.** 

An arrival coffee followed by a selection of 4 chosen oral presentations, each offering a 10-minute talk followed by a 5-minute discussion - highlighting emerging research and fresh perspectives across the field.

Session 9 10:00 - 12:15PM

**Established & Novel Therapies for Neurotransmitter Disorders.** 

This key session explores current treatments & groundbreaking advances, featuring two important discussions on gene therapy for AADC deficiency. A short coffee break is included- don't miss these critical updates shaping the future of care.

Session 10 12:15 - 13:30PM

Neurotransmitter Workshop for Patient & Public Engagement

Join us for an interactive session bringing together clinicians, researchers, and families to explore diagnostics, prognostics, therapies and patient-driven priorities in neurotransmitter disorders.

"This workshop has been made possible in part by grant 2024-347839 from the Chan Zuckerberg Initiative DAF, an advised fund of Silicon Valley Community Foundation."

### Researcher Prizes & Close 13:30 - 13:45PM

Celebrate the presentation of early career researcher prizes for best oral presentation and best poster

### Lunch & Depart from 13:45PM

Enjoy a final lunch together before making your way home.

# BIG Ideas, BOLD Research, all in just 60 minutes!

Don't MISS it...



Join us for 4 x Rapid-Fire Oral Presentations
10 minutes of Insight and 5 minutes of Discussion

# Meet the Experts Behind the Programme

INDConf 2025 brings together an extraordinary group of clinicians, researchers, and advocates who are shaping the future of care and discovery in neurotransmitter disorders. Below you'll find the individuals whose expertise and insight form the foundation of this programme. Full session details and timings will be shared closer to the event..

### Professor Manju Kurian

Neurotransmitter Disorders: Clinical Overview of Neurotransmitter Disorders

### Professor Keith Hyland & Professor Simon Heales

Keynote lecture: A Lifetime of Discovery in Neurotransmitter Disorders

### Professor Thomas Opladen

The iNTD Registry: A 2025 update

### Dr Alpa Dherai

Analytical Method Validation for CSF Monoamine HPLC Analysis: Experience from an Indian Centre.

### Professor Rafa Artuch

CSF HVA and 5-HIAA in the Pediatric Population: Clinical Impact of Continuous Reference Intervals.

### Dr Simon Pope

The Utility of Mass Spectrometry in CSF Metabolite Biomarker Analysis





# Meet the Experts Behind the Programme

#### Dr David Bick

UK Newborn Genomic Screening for Neurotransmitter Disorders: The Generation Study

### Professor Nenad Blau

The Spectrum of DDC variants in AADC deficiency: Insights into Genetic Mechanisms

### Dr Reem Alkhater & Dr Birgit Assmann

The Emerging Role of Monoamine Transportopathies in Human Disease

#### Professor Yair Anikster

DNAJC12 deficiency: Expanding Genotype, Phenotype and Emerging Disease Mechanisms

#### Professor Simon Heales

Secondary Neurotransmitter Disorders: Laboratory Insights

### Dr Tessa Wassenberg

Secondary Neurotransmitter Disorders: Clinical Phenotypes

### Professor Nick Greene & Dr Simon Pope

Disorders of Cerebral Folate Deficiency: Genotypes, Phenotypes, Pathogenesis, Laboratory

### Professor Peter Clayton & Professor Philippa Mills

Disorders of B6 Metabolism: Genotypes, Phenotypes, Pathogenesis, Laboratory

# Meet the Experts Behind the Programme

### Professor Phillip Pearl

Translational Arc for GABA Disorders

### Professor Johan Van Hove

An Update on Glycine Metabolism

### Professor Mita Bertoldi

In Silico and in Vitro Modelling for AADC Deficiency: Functional Insight into Disease Mechanisms

#### Dr Serena Barral

Advanced iPSC-based Disease Modelling for Neurotransmitter Disorders

### Professor Jo Ng

Preclinical Novel Therapy Development for Primary and Secondary Neurotransmitter Disorders

#### Dr Roser Pons

Treatable Neurotransmitter Disorders

### Dr Sam Shribman

Treating Adults with Neurotransmitter Disorders

### Professor Thomas Opladen

Gene Therapy for AADC deficiency: Long Term Outcomes with Putaminal Targeting.

### Dr Toni Pearson

Gene Therapy for AADC deficiency: Long Term Outcomes with Midbrain Targeting



"Behind every topic, every session and every speaker is a shared committment to families living with neurotransmitter disorders.

We invite you to be part of INDConf 2025 – a place where science meets support and every voice matters."





# THANKYOU

**Conference Co-ordinator**Lisa Flint

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Insta: #indconf2025



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