

# Conference Agenda

## Tuesday 10th September

Pembroke College, Oxford

**16.00 Registration**

**17.00 Opening Remarks**

*Professor Matthew Wood, Director, Oxford-Harrington Rare Disease Centre*

**17.15 Keynote Lectures**

**The Harrington Discovery Institute**

*Jonathan Stamler, President, Harrington Discovery Institute*

**Gene therapy for Retinitis Pigmentosa**

*Robert MacLaren, Professor of Ophthalmology, Nuffield Department of Clinical Neurosciences, University of Oxford*

**Gene therapy for Cystic Fibrosis**

*Eric Alton, Chair in Gene Therapy, Faculty of Medicine, National Heart & Lung Institute, Imperial College London*

**19.00 Drinks and canapés reception**

## Wednesday 11th September

**9.00 Session 1**

**Keynote Lecture – NIH National Center for Advancing Translational Sciences**

*Chris Austin, Director, NIH NCATS*

**Title tbc**

*Rita Balice-Gordon Global Head, Rare and Neurological Diseases Therapeutic Area, Sanofi*

**Title tbc**

*Thomas Voit, Director of the NIHR Great Ormond Street Hospital (GOSH) BRC, Professor and Honorary Consultant of Paediatrics at GOSH*

**Title tbc- new drug targets in Friedreich's Ataxia**

*Richard Wade-Martins, Professor of Molecular Neuroscience, Department of Physiology, Anatomy and Genetics, University of Oxford*

**Title tbc**

*Kevin Talbot (tbc), Professor of Motor Neuron Biology, Nuffield Department of Clinical Neurosciences, University of Oxford*

**11.30 Coffee**

**Session 2**

**Title tbc**

*Francesco Muntoni, Chair of Paediatric Neurology, UCL*

**Gene therapy in Duchenne Muscular Dystrophy and Spinal Muscular Atrophy**

*Laurent Servais, Department of Paediatrics, University of Oxford*

**Session 3**

**Title tbc In born errors of metabolism**

*Tim Cox, Department of Medicine, University of Cambridge*

**Title tbc**

*Wyatt Yue, Structural Genomics Consortium, University of Oxford*

**Title tbc**

*Atul Chopra, Investigator, Harrington Discovery Institute, University Hospitals, Cleveland*

**Title tbc**

*Bob Lightowlers, Professor of Molecular Neuroscience, University of Newcastle*

**13.30 Lunch**

**14.15 Session 3**

**Rare blood disorders**

*Mike Laffan, Imperial College*

**Title tbc**

*Georg Holländer, Hoffmann and Action Medical Research Professor of Developmental Medicine*

**Title tbc**

*Bobby Gaspar, CSO, Orchard Therapeutics*

**Session 4**

**Title tbc Gene therapy in Dermatology**

*John McGrath, King's College London*

**Title tbc**

*Stuart Hughes, Vertex Pharmaceuticals*

**Title tbc**

*Michael Panzara, Chief Medical Officer, Wave Life Sciences*

**16.20 Coffee break**

**16.45 Session 4**

**100k Genomes Project**

*Richard Scott, Clinical Lead, Rare Diseases, Genomics England*

**Title tbc**

*Lucy Raymond, Professor of Medical Genetics and Neurodevelopment, Department of Medical Genetics, University of Cambridge*

**Title tbc**

*Jenny Taylor, Wellcome Trust Centre for Human Genetics, University of Oxford*

**Title tbc**

*Henry Houlden, Consultant Neurologist, UCL and Head of Neurological GeCIP Domain, Genome England*

**Title tbc**

*Hugh Watkins, Radcliffe Professor of Medicine, University of Oxford*

**Title tbc**

*Tim Barrett, Leonard Parsons Professor of Paediatrics and Child Health, University of Birmingham*

**Conference residential dinner**

**Thursday 12th September**

**Pembroke College, Oxford**

**8.30 Registration**

**9.00 Session 5**

**Title tbc**

*Seng Cheng, CSO Rare Diseases, Pfizer*

**Title tbc**

*Mahindra Makhija, Takeda Pharmaceuticals*

**Title tbc**

*Per Lundin, Chief Operating Officer, Evox Therapeutics*

**Title tbc**

*Steve Murray, Mallinckrodt Pharmaceuticals*

**Title tbc**

*Madhurima Benekareddy, Project Lead and Lab Head, Roche*

**11.00 Coffee Break**

**11.20 Session 6**

**Birmingham Rare Disease Network**

*David Adams, Director, NIHR Birmingham Biomedical Research Centre, University of Birmingham*

**Title tbc**

*Willem Owehand, Professor of Experimental Haematology, University of Cambridge*

**LifeArc Philanthropic Fund for Rare Disease Translational Research**

*Catriona Crombie, Philanthropic Fund Manager, LifeArc*

**12:45 Closing Remarks and Poster Prize**