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

16.15  **Coffee break**

16.35  **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

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**Conference residential dinner**

**Thursday 12th September**

**Pembroke College, Oxford**

8.30  **Registration**

9.00  **Session 5**

**Emerging therapies for hemophilia and sickle cell anemia**  
Seng Cheng, CSO Rare Diseases, Pfizer

**Title tbc**  
Mahindra Makhija, Director, Translational Research, Rare Disease Unit, Takeda Pharmaceuticals

**Title tbc**  
Per Lundin, Chief Operating Officer, Evox Therapeutics

**Title tbc**  
Steve Murray, VP Strategy and Innovation, 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