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

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