



Gene Therapy for Inherited Metabolic Disease Meeting, Thursday 8 March 2012, UCL Institute of Child Health, London

Dear Colleague,

We would like to invite you to register for the [Gene Therapy for Inherited Metabolic Diseases meeting](#), which will be held the day before the main BSGT Conference on Thursday 8th of March, 2012. Separate [registration](#) is required for this satellite meeting and is not included for those already registered for the main conference on the 9th March. The meeting, the first of its kind dedicated specifically to Inherited Metabolic Diseases, will be held at the UCL Institute for Child Health in central London and aims to bring clinicians and researchers together to debate application and promotion of novel therapies for these disorders.

Conference Information

The venue has been chosen because UCL is one of the world leaders in gene therapy, with several groups running successful clinical trials. Now is the ideal time to decide which patient groups could benefit most from gene therapy and to share the expertise to expedite treatments to the clinic.

Provisional Programme

Registration open

Speaker

- 10:00 Overview of Gene therapy - Steven Howe (UCL)
- 10:20 Systemic Gene Delivery - Simon Waddington (UCL)
- 10:40 Organic acidaemias - Chuck Venditti (NIH)
- 11:10 OTC deficiency - Ian Alexander (Sydney)
- 11:40 Gene delivery to brain - Ahad Rahim (UCL)
- 12:00 Lunch
- 13:00 Neurometabolic disorders: need for treatments - Manju Kurian (UCL)
- 13:20 AADC clinical trial- Krystof Bankiewicz (UCSF)
- 13:50 Clinical trial ALD - Nathalie Cartier (Inserm)
- 14:20 Lysosomal storage disorders: need for new treatments - Tim Cox (Cambridge)
- 14:50 Coffee break
- 15:20 Gene therapy for MPS3 - Brian Bigger (Manchester)
- 15:50 Gene therapy for MLD/NCL/MPS1 - Dolan Sondhi (Cornell)
- 16:20 Gene therapy for NCL - Mark Sands (Washington University in St. Louis)
- 16:50 General discussion - targets? Tools?
- 17:20 End

If you have any questions about the event, please contact Clare Beach at office@bsgt.org
We look forward to welcoming you to the meeting in March.

With best regards

Paul Gissen, Emma Footitt, Sara Mole and Steven Howe. Local organising committee.