

BSGT 2012 Provisional Programme.
UCL Institute of Child Health 8th March
Satellite meeting:
Gene therapy for inherited metabolic disease

09:00	Registration open	
10:00	Opening remarks	Paul Gissen (UCL)
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, Paris)
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)
16:50	General discussion – targets? Tools?	
17:30	End / Networking	