Surveillance of BPSU Rare Childhood Disease

Birmingham Children's Hospital NHS
NHS Foundation Trust

From bench to bedside: New treatments for children with rare disease

Monday 27th February 2017, Birmingham Children's Hospital

	Programme overview
08.45-09.30	Registration, tea & coffee
Morning chair	**Dr Larissa Kerecuk,** *Rare Disease Lead, Birmingham Children's Hospital NHS Foundation Trust*
09.30-09.40	**Leading the way in the delivery of paediatric care for those with a rare disease** *Dr Sarah Jane Marsh, Chief Executive Officer, Birmingham Children's Hospital NHS Foundation Trust*
09.40-10.00	**Integrating research into everyday care - Birmingham Children's Hospital experience** *Dr Larissa Kerecuk, Rare Disease Lead, Birmingham Children's Hospital NHS Foundation Trust*
10.00-10.20	**A history of medical invention** *Alastair Kent OBE, Rare Disease UK*
10.20-10.40	**TITLE TO BE CONFIRMED** *Professor Timothy Barrett, Paediatric programme lead for the NIHR Rare Disease - Translational Research Consortium, University of Birmingham*
10.45-11.15	**Coffee break**
11.15-11.35	**Implementation of the UK strategy for rare diseases** *Ben Howlett MP, Chair of the All Party Parliamentary Group on rare, genetic and undiagnosed conditions*
11.35-12.00	**Immuno-therapy, a new way to treat cancers** *Dr Frank Mussai, Clinical Senior Lecturer in Paediatric Oncology, University of Birmingham*
12.00-12.25	**TITLE TO BE CONFIRMED** *Professor Bobby Gaspar, Professor of Paediatrics and Immunology, Great Ormond Street Hospital*
12.25-12.45	**Key note speaker** **Dr Gina Radford,** **Deputy Chief Medical Officer for England & Rare Disease Champion**

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12.45-14.00	**Lunch**
Afternoon chair	**Dr Richard Reading,** *Chair of the British Paediatric Surviellance Unit*
14.00-14.10	**Harnessing research to deliver innovative treatments** *Professor David Adams, Birmingham Health Partners*
14.10-14.30	**Future perspectives: Moving towards Batten Disease treatments** *Dr Sara Mole, Reader in Molecular Cell Biology,* *University College London*
14.30-14.50	**Trials and tribulations of delivering eculizumab** *Dr Sally Johnson, Consultant Paediatric Nephrologist,* *University of Newcastle*
14.50-15.10	**Emerging therapeutic opportunities for lysosomal disorders** *Professor Timothy Cox, Consultant Physician,* *University of Cambridge*
15.10-15.30	**Will discovering the AHI1 gene lead to improved treatment for Joubert syndrome** *Professor John Sayer, Clinical Professor of Renal Medicine,* *University of Newcastle*
15.30-16.00	**Coffee break**
16.00-16.20	**Providing quality care for patients with rare disease** *Sheela Upadhyaya, Associate Director, Highly Specialised Technology Program - NICE*
16.20-16.35	**Funding rare disease research post-Brexit** *Louise McCathie, Director of Fundraising* *Birmingham Children's Hospital NHS Foundation Trust*
16.35-16.55	**TITLE TO BE CONFIRMED** *Kay Parkinson, Director Cambridge Rare Disease Network*
16.55	Close