

**Westminster Health Forum policy conference:  
Priorities for rare disease diagnosis, care and treatment in England**

Timing: Morning, Tuesday, 16<sup>th</sup> July 2024

\*\*\*Taking place online\*\*\*



**WESTMINSTER  
HEALTH FORUM**

Agenda subject to change

- 8.30 Registration
- 9.00 **Chair's opening remarks**  
**Baroness Neville-Jones**
- 9.05 **Assessing the current landscape for the treatment of rare diseases - progress of the England Rare Diseases Action Plan 2024**  
**Professor Timothy Barrett**, Director, Centre for Rare Disease Studies, University of Birmingham  
Questions and comments from the floor
- 9.30 **Progress and next steps for newborn screening for early detection and diagnosis of rare diseases**  
**Dr Graham Shortland**, Vice-Chair, UK National Screening Committee
- 9.40 **Improving the delivery of care for patients with rare diseases**  
*developing effective care pathways | patient accessibility to care | specialised commissioning | transitions from paediatric to adult care | coordinated care services | implementing a holistic approach to care | Early Access to Medicines Scheme*  
**Adam Galloway**, Clinical Academic and Children's Physiotherapist, Leeds Children's Hospital  
**Charlotte Chapman-Hart**, Rare Disease Patient Representative and Business Transformation Officer, North West Anglia NHS Foundation Trust  
**Dr Paul Clift**, Clinical Lead, Centre for Rare Diseases, Queen Elizabeth Hospital Birmingham  
**Professor Derralynn Hughes**, Clinical Director, Research and Innovation, Royal Free London NHS Foundation Trust  
**Professor Paramala Santosh**, Professor, Developmental Neuropsychiatry and Psychopharmacology, Kings College London
- 10.10 Questions and comments from the floor
- 10.35 **Approaches to workforce education for rare diseases and therapies - raising awareness among healthcare professionals to improve diagnosis and treatment**  
**Dr Lucy McKay**, CEO, Medics4RareDiseases  
Questions and comments from the floor
- 11.00 **Chair's closing remarks**  
**Baroness Neville-Jones**
- 11.05 Break
- 11.15 **Chair's opening remarks**  
**Dr Victoria Lund**, Programme Manager, Respiratory, Inflammation and Rare Diseases, Medical Research Council
- 11.20 **Priorities for regulation and licencing pathways for innovative treatments**  
**Dr Lincoln Tsang**, Partner, Ropes and Gray
- 11.30 **Advancing research and innovation across the rare disease pathway**  
*exploring the future potential of genomic medicine | clinical trials | next steps for children's treatments through the Rare Therapies Launch Pad | improving research eco-system collaboration and the role of the Rare Disease Research UK Platform | coordinating data and disease registries | licencing and regulation pathways*  
**Catriona Crombie**, Head, Rare Disease, LifeArc  
**Deborah Richards**, UK General Manager, Alexion  
**Dr Victoria Lund**, Programme Manager, Respiratory, Inflammation and Rare Diseases, Medical Research Council  
**Professor Simon Johnson**, Professor, Respiratory Medicine, University of Nottingham  
**Dr Lucy Allen**, Director, Research and Healthcare Data, Cystic Fibrosis Trust
- 12.00 Questions and comments from the floor
- 12.30 **Next steps for utilising innovation in genomics for care and treatment of rare diseases**  
**Ana Lisa Tavares**, Clinical Lead, Rare Disease Research, Genomics England  
Questions and comments from the floor
- 12.55 **Chair's and Westminster Health Forum closing remarks**  
**Dr Victoria Lund**, Programme Manager, Respiratory, Inflammation and Rare Diseases, Medical Research Council  
**Jessica Lear**, Westminster Health Forum