

What rare diseases research topics would you like to hear about in future events?

Mitochondrial diseases	Drug repurposing
Inborn errors of metabolism	Autoinflammatory
Autism / Epilepsy	Reimbursement and market access
Progress in gene therapies	Genetic Kidney Diseases / Cholangitis
Scientific basis of rare diseases /research progress	immune system failures
Recent progress made in treatment/ identification	Treatments, cures available, personalised medicine linked to rare diseases
Follow a journey of a potential treatment into being part of the NHS	Immunodeficiency / Causes of Autoimmune Disease
How to set up a Scientific Advisory Board for small rare groups/ How to find researchers	Genetics- consent/implications/complications DCA haematology conditions
Children's diseases, cancers, different varieties of rare diseases	Therapeutics aspects of rare diseases (less genomics, genetics)
Behçet's and diseases that develops during childhood - how they impact on daily living	Ethics of genomic medicine
Issues patients face in accessing health and social care support	Inherited blood diseases, rare cancers, rare infections
An example of a novel therapy that has reached the clinic and any learnt outcomes	NIHR BioResource trials update (numbers that are active/ about different participants involved /what sorts of trials are they)
Updates on current studies (interested in SAPHO)	Motor neurone disease

Suggestions

- A mix of clinicians and patient groups speakers (hear patient experience and the impact of results)
- Rare patient/ patient support group representative in the audience may want to hear more about the advances in diagnosing their 'rare disease' of interest
- I'd like to hear from patient advocacy groups in the field, how they raise awareness and what they'd like others to do
- Highlight projects with strong patient involvement- what patient groups can bring to a research setting

Comments

- Subjects had great access to non-medical and info for a medical audience too
- Voting kept it interactive which was great
- Importance of educating the health service in rare diseases
- Projects with strong patient involvement- highlighting what patients' groups can bring to a research setting. Hopefully make more researchers open to it and continue motivation for patient groups