NIHR | BioResource

'Think Research' Rare Diseases Patient Day

Thursday 26 March 2020 at Friends House, London

10:00	Registration in the North Lobby and refreshments and networking in East Lobby		15 minutes
10:15	Welcome Ring20 research & support UK	**Allison Watson**: How our small patient group is working with others to drive change	The Light
10:25	NIHR BioResource	**Prof Patrick Chinnery**: NIHR BioResource and new rare disease studies	
10:40	Department of Health and Social Care	**Dr Monika Preuss**: How diagnostic developments in research is helping shape policy	
10:55	Cambridge	**Prof Nick Morrell:** Genetic diagnoses in patients with pulmonary arterial hypertension	
11:10	UCL and Moorfields	**Prof Andrew Webster:** Empowering patients: how the rare eye disease community is collecting, managing and sharing their data for research	
11:25	Short comfort break	Restrooms are located in the West Lobby	

11:30	Workshops (hot drinks will be available in each of the rooms)			50 minutes
	Room X	Room X	Room X	Room X
	Communicating rare disease stories and research Creating compelling stories and translating the science for different audiences, with Department of Public Health & Primary Care, UoC & PAWS-GIST	**Research using health data - promises and privacy** Why data is central to research, and how technology will help us understand rare diseases in the future, with IT Director, NIHR BioResource	**Keeping up to date with the latest research** How to find research on conditions, treatments & study outcomes, with University of Cambridge Medical Library & TSA Association	**What makes a good research question?** Develop an answerable research question, with Patient Led Research Hub & PKD Charity

12:20	Lunch - one hour	Refreshments, Discussions, Displays & Networking	East Lobby
12:50	**Optional Discussions**	Group discussions about **'Funding for Research'** and **'Fundraising ideas'** held in the last 30 minutes of the lunch break. This is an opportunity to chat to fellow members whilst eating your lunch. Feel free to join one of these groups	The Light

13:20	Workshops (water will be available in each of the rooms)			50 minutes
	Room X	Room X	Room X	Room X
	Communicating rare disease stories and research	**Research using health data - promises and privacy**	**Keeping up to date with the latest research**	**What makes a good research question?**

14:10 **Expert Panel on Additional Findings in Research:** Q&A session	**"There is no consensus in the research community on feedback to participants on additional findings"** **Would you want to know?** There will be a short introduction to this session and in the Q&A we'll cover: • What **do** rare disease patients want to know – their right **not** to know • The family's right to know – including if the patient dies • Findings we are uncertain or cannot do anything about - have no treatment • Risk factors - a potential future health risk, sensitivity to certain drugs • Paternity / reproductive / genetic issues and risks which could impact family members • How and when to communicate these to patients and families	The Light

15:10	Break Tea & Coffee	Refreshments & Networking	East Lobby
15:20	NICE, Centre for Health Technology Evaluation	**Sheela Upadhyaya:** How NICE use Highly Specialised Technology evaluations to recommend new treatments for rare diseases	The Light
15:40	Genetic Alliance	**Dr Amy Hunter: "**Living with a Rare Condition: the effect on Mental Health"?	
15:55	Open Targets	**Ian Dunham:** Drug discovery: identifying and prioritising targets for drug development	
16:15	Reprocell	**Dr Karen McAulay:** Creating models of rare disease using patients samples	
16:25	NIHR BioResource	**Prof John Bradley:** Closing remarks, the event ends 16:30	

Find details on providing a 'Patient Statement' on https://cambridgebrc.nihr.ac.uk/thinkresearch/