

## **Applied Clinical Research on Very Rare Diseases (2012)**

In 2012, the NIHR issued a call for research into interventions for Very Rare Diseases.

For the purpose of the call, a very rare disease is one that affects less than 1 in 100,000 of the general population, particularly those whose specialist care is provided through centres funded by the National Specialised Commissioning Group (NSCG) and equivalent arrangements from April 2013.

**Status:** Closed

**Date opened:** October 2012

**Date closed:** March 2013

### **The following NIHR managed research programmes participated:**

- Efficacy and Mechanism Evaluation (EME)
- Health Services and Delivery Research (HS&DR)
- Health Technology Assessment (HTA)
- Invention for Innovation (i4i)
- Programme Grants for Applied Research (PGfAR)
- Research for Patient Benefit (RfPB)

## Funding Outcomes

Research Programme	NIHR reference	Application Title	Lead Applicant
EME	12/205/46	Optimal utilisation of biologic drugs in Behçet's Disease: a randomised controlled trial of infliximab vs alpha interferon, with genotyping and metabolomic profiling, towards a stratified medicines approach to treatment.	Prof Robert Moots (University of Liverpool)
EME	12/205/56	Treatment of Barth Syndrome by CARDIOlipin MANipulation (CARDIOMAN): A randomised placebo-controlled pilot trial conducted by the nationally commissioned Barth Syndrome Service	Dr Colin Steward (University Hospitals Bristol NHS Foundation Trust)
EME	12/205/36	A randomised trial comparing surgical and radiotherapy strategies enabling rational treatment selection for primary Merkel Cell Carcinoma (MCC), a rare virus and immune-related aggressive skin cancer	Dr Neil Steven  University of Birmingham
EME	12/205/28	COHESIVE - COrticosteroids in HErpes SImplex Virus Encephalitis	Prof Tom Solomon (University of Liverpool)
HTA	12/173/14	Radiation versus Observation following surgical resection of Atypical Meningioma: a randomised controlled trial (The ROAM trial).	Mr Michael D Jenkinson (The Walton Centre NHS Foundation Trust, Liverpool)

PGfAR	RP-PG-1212-20009	Developing a psychological intervention to improve ultraviolet protection and clinical outcomes in Xeroderma Pigmentosum	Dr Robert Sarkany (Guy's and St Thomas' NHS Foundation Trust)
RfPB	PB-PG-1112-29056	Assessment and satisfaction of services for Juvenile Huntington's Disease and modelling alternative methods of service organisation	Dr Oliver Quarrell (Sheffield Children's NHS Foundation Trust)
RfPB	PB-PG-1112-29122	Systematic reviews of treatments for children's cancer – central nervous system tumours	Ms Jayne Wilson (Birmingham Children's Hospital NHS Foundation Trust)

