

## Adult and Paediatric Haemoglobinopathy Services

## Haemoglobinopathy and Rare Inherited Anaemia (RIA) Open Clinical Studies and Trials Wessex and Thames Valley Networks

Study/ Trial Name	PI	Brief Description	Sites	Link
HIBISCUS trial <b>Sponsor:</b> Forma Therapeutics, Inc.	Dr Wale Atoyebi	This clinical trial is a Phase 2/3 study that will evaluate the efficacy and safety of etavopivat and test how well etavopivat works compared to placebo to improve the amount of hemoglobin in the blood and to reduce the number of vaso-occlusive crises (times when the blood vessels become blocked and cause pain).	Oxford	<a href="https://clinicaltrials.gov/ct2/show/NCT04624659">https://clinicaltrials.gov/ct2/show/NCT04624659</a>
The Molecular Investigation of Unexplained Anaemia Study <b>Sponsor:</b> Oxford University Hospitals NHS FT	Dr Noemi Roy	Pure laboratory-based study covering patients with suspected RIA or/ and their relatives. Aims to identify new genes (or new mutations in known genes) that are involved in erythropoiesis.	Oxford Milton Keynes Basingstoke Winchester Southampton	<a href="https://www.ouh.nhs.uk/services/referrals/genetics/genetics-laboratories/rare-disease-genomics/non-malignant-haematology/rare-anaemia.aspx">Health Research Authority https://www.ouh.nhs.uk/services/referrals/genetics/genetics-laboratories/rare-disease-genomics/non-malignant-haematology/rare-anaemia.aspx</a>
NIHR BioResource: Rare Diseases (Biobank) <b>Sponsors:</b> The University of Cambridge and Cambridge University Hospitals NHS Foundation Trust	Dr Noemi Roy	Collecting samples from patients with rare disease e.g Haemoglobinopathy Disorders (Sickle Cell Disease, Thalassaemia), aiming to identify genetic causes of rare diseases, to improve diagnosis and to support work to develop and validate treatments.	Oxford	<a href="https://bioresource.nihr.ac.uk/using-our-bioresource/our-cohorts/rare-diseases-bioresource/">https://bioresource.nihr.ac.uk/using-our-bioresource/our-cohorts/rare-diseases-bioresource/</a>
RUDY study	Dr Noemi Roy	Rudy is a study in Rare diseases aiming to transform clinical care for participants through patient driven research	Oxford	<a href="https://rudystudy.org/">https://rudystudy.org/</a>

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Investigating the Quality of Life Experiences in Patients with Sickle Cell Disease <b>Sponsor:</b> University of Oxford				
Experiences of Young Migrants with Sickle Cell Disease Study <b>Sponsor:</b> University of Nottingham	Dr Brenda Poku (Nottingham University)	The study aims to understand the unique experiences of migrant children and young people with sickle cell disease to help guide future service provision	Oxford	<a href="https://www.hra.nhs.uk/planning-and-improving-research/application-summaries/research-summaries/experiences-of-young-migrants-living-with-sickle-cell-disease/">https://www.hra.nhs.uk/planning-and-improving-research/application-summaries/research-summaries/experiences-of-young-migrants-living-with-sickle-cell-disease/</a>
Sickle Eye Project (National) Retinopathy Screening in Sickle Cell Disease study <b>Sponsors:</b> London North West Healthcare NHS Trust and funded by National Institute for Health and Care Research; Roche.	Professor Susan Downes (Oxford Univ. Hospitals, NHS FT)	Study to identify negative impact on vision due to sickle cell disease and how this affects patients. The information from the study will be used to design improved eye health care for people with sickle cell disease.	Oxford	<a href="https://beartofresearch.nihr.ac.uk/trial-details/trial-detail?trialId=50418&amp;location=Coventry&amp;distance=#">https://beartofresearch.nihr.ac.uk/trial-details/trial-detail?trialId=50418&amp;location=Coventry&amp;distance=#</a>