South Thames Sickle Cell & Thalassaemia Network

Actively recruiting trials in the STSTN Network

A. STAND Clinical Trial

Crizanlizumab is a new medication that was licensed by the Food and Drug Administration (FDA) for use in sickle cell patients who live in the USA in 2019. It has been shown in clinical trials to reduce the pain episodes per year by 45%. This treatment works in sickle cell by reducing the ability of blood cell to stick to the blood vessel's walls. At the present time Crizanlizumab is unlicensed for use in Europe and the United Kingdom.

The STAND clinical trial is a project which is comparing the effectiveness of 2 different doses of Crizanlizumab in reducing Sickle cell pain episodes when compared to standard sickle cell care (placebo arm). This is an international multicentre project that is aiming to recruit a total of 240 patients who will be randomly assigned to one of the 3 treatment arms (5mg dose, 7.5mg dose or standard care). Patients participating will attend for monthly infusions of the treatment for the duration of the trial.

To take part in this trial you must have a confirmed diagnosis of sickle cell, be aged at least 12 years and not be on a transfusion program. Patients on Hydroxycarbamide/Hydroxyurea can enter the trial. Please speak to your haematologist or sickle cell doctor if you are interested in taking part, should you fit the entry criteria you can be referred to the sickle cell teams at the centres listed below where patients are being actively recruited and you can have further discussion and be considered for entry onto the trial.

Adult sickle participants are being recruited at:

1. Guys and St Thomas NHS Foundation Trust
2. Kings College Hospital NHS Foundation Trial

Children with sickle cell are being recruited at:

1. Evelina Children's Hospital
2. Kings College paediatric sickle cell unit

B. STEDFAST Clinical Trial

Crizanlizumab is a new medication that was licensed by the Food and Drug Administration (FDA) for use in sickle cell patients who live in the USA in 2019. It has been shown in clinical trials to reduce the pain episodes per year by 45%. This treatment works in sickle cell by reducing the ability of blood cell to stick to the blood vessel's walls. At the present time Crizanlizumab is unlicensed for use in Europe and the United Kingdom.

Kidney function in sickle cell patients is affected from an early age and the incidence of kidney impairment increases with age such that a fifth of all adults with sickle cell disease have kidney impairment.

The STEADFAST clinical trial is looking to see if treatment with Crizanlizumab will slow or stop the progression of this kidney damage. Patients on this clinical trial will be randomly assigned to either continue on standard sickle treatment or receive monthly infusions with Crizanlizumab. This is trial is also an international trial with patients participating in centres in the USA, France and UK amongst others, 170 patients will participate in total.

To take part in this trial you must have a confirmed diagnosis of sickle cell, be aged at least 16 years and not be on a transfusion program. Patients on Hydroxycarbamide/Hydroxyurea can enter the trial. Please speak to your haematologist or sickle cell doctor if you are interested in taking part, should you fit the entry criteria you can be referred to the sickle cell teams at the centres listed below where you can have further discussion and be considered for entry onto the trial.

Adult (Aged 16+) sickle participants are being recruited at:

1. Guys and St Thomas NHS Foundation Trust
2. Kings College Hospital NHS Foundation Trial

C. SOLACE Clinical Trial, Phase III trial in children.

Crizanlizumab is a new medication that was licensed by the Food and Drug Administration (FDA) for use in sickle cell patients who live in the USA in 2019. It has been shown in clinical trials to reduce the pain episodes per year by 45%. This treatment works in sickle cell by reducing the ability of blood cell to stick to the blood vessel's walls. At the present time Crizanlizumab is unlicensed for use in Europe and the United Kingdom.

The efficacy and safety of crizanlizumab has been demonstrated in adults with sickle cell disease in previous research studies, the SOLACE clinical trial is a project aiming to confirm and establish appropriate dosing in children, it will also evaluate the safety of crizanlizumab in children with sickle cell aged 6 months to <18 years. Participants in this trial can be on Hydroxycarbamide (Hydroxyurea), or not and they must have had at least one pain episode in the past year. Patients on transfusions cannot take part in this trial.

The study will include at least 100 children, all of whom will receive Crizanlizumab which will be supplied as open label medication. All patients participating will attend in week 1, week 3 then monthly for infusions of the treatment for the duration of the trial.

Please speak to your haematologist or sickle cell doctor if you are interested in taking part, should you fit the entry criteria you can be referred to the sickle cell teams at the centres listed below where you can have further discussion and be considered for entry.

This trial is recruiting children at

1. Evelina Children's Hospital
2. Kings College paediatric sickle cell unit

D. GBT440 - 29

Voxelotor is a medication developed by a company in the USA called Global Blood Therapeutics (GBT), it is an oral medication that is now licensed for use in sickle patients in the USA. It was shown in clinical trials to improve the anaemia and other day to day symptoms in adults and adolescents with sickle cell. Voxelotor is not presently licensed for use in the UK and Europe. This clinical trial is investigating how well higher doses of Voxelotor will be tolerated in patients with sickle cell. In total 45 patients will participate in the trial.

You can participate in this trial if you are aged between 18 to 60 years old and have HbSS or HbSb0 sickle cell. Please speak to your haematologist or sickle cell doctor if you are interested in taking part, should you fit the entry criteria you can be referred for further discussion and be considered for entry onto the trial.

Adult (Aged 18+) sickle participants are being recruited at:

1. Guys and St Thomas NHS Foundation Trust

E. TAPS2: Transfusion (Antenatally) in Pregnant Women With SCD

Pregnant women with sickle cell disease have an increased risk of both sickle and pregnancy complications, including raised blood pressure. Their babies may grow more slowly in the womb, are more likely to be born early and need special care and have a higher risk of dying.

The only treatments currently available for women with SCD are Hydroxycarbamide (which cannot be used during pregnancy) and blood transfusion. Currently, blood transfusion is only used during pregnancy to treat emergency complications. It has been suggested that giving blood transfusions throughout pregnancy could improve outcomes for both mother and babies.

In an Exchange Blood Transfusion (EBT), sickle blood is mechanically removed and simultaneously replaced with donor red cells. A trial is needed to assess if EBT given every 6-10 weeks, starting before 18 weeks of pregnancy, compared to standard care will result in improved outcomes for pregnant sickle women.

The TAPS2 is a feasibility being carried out at multiple maternity centres in England which is assessing the willingness of eligible pregnant women with sickle cell to join such a study and if they find the intervention acceptable.

This trial will recruit 50 women and allocate them randomly to either EBT or standard care throughout their pregnancy.

Pregnant sickle women (Aged 18+) are being recruited at:

1. Kings College Hospital
2. Guy's and St Thomas NHS FT

F. INV543

Hydroxycarbamide (Hydroxyurea) was for many years the only licensed mediation for patients with sickle cell disease, however it is a medication that is usually formulated as a capsule or tablet. This clinical trial is investigating the safety of a new oral solution (liquid) of Hydroxycarbamide in children with sickle cell. It is a single arm study so all participants will get the new liquid preparation of Hydroxycarbamide (Hydroxyurea).

To take part in this trial you have to be aged over 6 months but under 18 years and have sickle cell. Please speak to your haematologist or sickle cell doctor if you are interested in taking part, should you fit the entry criteria you can be referred for further discussion and be considered for entry onto the trial.

Children (Age 6 months – 17.99 years) with sickle cell are being recruited at:

1. Kings College paediatric sickle cell unit
2. Evelina Children's Hospital

G. 'Impact of Roald Dahl Nurses on patient care and outcome'

The Roald Dahl Charity have commissioned Sheffield Hallam University to undertake a study to understand how the Roald Dahl's children's nurses are helping children and young people with chronic illnesses. King's College Hospital has been fortunate enough to have our haemoglobinopathy transition nurse specialist funded by this charity- the nurse undertakes vital lifesaving work for children with sickle cell disease who transition from children's to adult services at King's.

This study will involve completing a completely confidential short online survey by parents and young people with sickle cell disease or thalassaemia between ages 16-24 about whether the nurse's role has helped them transition their care from children's to adults services. The information obtained from this survey is only available to the research team at the Sheffield Hallam University and no patient identifiable information will be used. The study has been reviewed and approved by an Ethics Committee.

Recruitment to the study runs from *September to November 2020* and we aim to collect 40 responses from parents and patients by the end of the study. A report will be available to share in this website at the end of the study in late 2021.

If you want to take part in this study please contact Giselle Padmore-Payne at giselle.padmore-payne@nhs.net

H. Bioresource

The National Institute for Health Research (NIHR) BioResource has been establishing a panel of thousands of volunteers with and without health problems from all over the country, this includes patients with sickle cell disease which is considered a rare condition in the UK. All volunteers are asked to donate a small blood sample (or sometimes saliva sample) and give consent to be contacted and invited to participate in future medical research studies, based on analysis of their samples and information they have supplied.
By recruiting thousands of volunteers with a rare disease in their family, the NIHRBR-RD aims to help with (1) the development of more affordable DNA-based tests for the diagnosis of rare diseases where the gene is known and (2) the discovery of genes causing rare diseases; currently only half of the genes for rare diseases are known.
Anonymised information and samples from the BioResource can be made available to researchers and doctors working in biomedical and healthcare research in both the public and private sector, in the UK and overseas.
Once the gene causing a rare disease has been identified, the search for better treatments can start. While not always successful, several rare diseases now have new treatments which have already dramatically improved care, giving hope that this will extend to many more in the future.

Anyone can take part in this study, so please speak to your haematologist or sickle cell doctor who can refer you for further discussion and entry onto the trial.

Any patient with sickle cell:

1. Children can be referred to Kings College Hospital NHS Foundation Trust
2. Adult patients should be referred to Guys and St Thomas NHS FT Sickle cell team.