ISRCTN17438123 https://doi.org/10.1186/ISRCTN17438123

A small pilot feasibility study for a possible randomised control trial comparing clinical outcomes and quality of life following two different transfusion strategies in children undergoing allogeneic hematopoietic stem cell transplant (HSCT)

Submission date 20/05/2019	**Recruitment status** No longer recruiting	[X] Prospectively registered [_] Protocol
Registration date 23/05/2019	**Overall study status** Completed	[_] Statistical analysis plan [X] Results
Last Edited 01/08/2025	**Condition category** Other	[_] Individual participant data

Plain English summary of protocol

Background and study aims

This is a small pilot feasibility study for a possible randomised controlled trial comparing clinical outcomes and quality of life following two different transfusion strategies in children undergoing allogeneic hematopoietic stem cell transplant (HSCT). Children who undergo allogeneic HSCT usually receive red blood cell transfusions in the initial period after their transplant, due to reduced haemoglobin levels as a result of bone marrow suppression. In this situation, the red cell transfusions are commonly given when the child's haemoglobin falls below 70-80g/L. The evidence for this practice is based largely on clinical trials which suggest that in most situations transfusing red cells at haemoglobin thresholds of 70g/L is sufficient, with no benefit of transfusing at higher haemoglobin thresholds. However, this evidence is largely from adult trials in non-HSCT patients. Clinical studies are required to help us understand the best strategy for use of red cell transfusions in paediatric HSCT.

Who can participate?

Patients aged between and including 1 and 17 years who are due to undergo allogeneic Haematopoietic Stem Cell Transplant and are expected to require red cell transfusions can participate.

What does the study involve?

Patients aged between and including 1 and 17 years will participate in this study for the first 100 days after their transplant. Patients will be randomised between the two red cell transfusion strategies: transfusing at a haemoglobin threshold of ≤ 65 g/L (Arm A) or ≤ 80 g/L (Arm B).

Participants of 8 years and older will be asked to fill in short questionnaires about their quality of life at certain intervals during the period of their study. All parents will be asked to fill in an equivalent parent questionnaire

What are the possible benefits and risks of participating?
Benefits: Patients in Arm B (higher haemoglobin threshold) may receive a few additional transfusions than they would have had if they had not participated in the trial and patients in Arm A (lower haemoglobin threshold) may receive slightly fewer transfusions.
Risks: As for all blood transfusions, there are small risks associated with having a transfusion. These risks are described in the national transfusion leaflets produced by NHS Blood and Transplant and SNBTS. This includes the very small risks of catching an infection, such as hepatitis B or HIV (the virus that causes AIDS). Other risks include allergic reactions or developing antibodies to the blood transfused. However, all the paediatric patients undergoing allogeneic HSCT participating in the trial will be expected to receive red cell transfusions and are likely to have had multiple transfusions previously, and so these are not new risks for them. NHSBT has a rigorous system for testing all blood donations for viruses such as hepatitis B or HIV.

Where is the study run from?
NHSBT Clinical Trials Unit, Cambridge, UK.

When is the study starting and how long is it expected to run for?
June 2019 to June 2023

Who is funding the study?
NHS Blood and Transplant, UK

Who is the main contact?
CTU@nhsbt.nhs.uk

Contact information

Type(s)
Scientific

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Additional identifiers

Integrated Research Application System (IRAS)
246398

Central Portfolio Management System (CPMS)
41935

Study information

Scientific Title
Red Cell Transfusion in Paediatric Allogeneic HSCT

Acronym
RePAST

Study objectives
The principal research question is to see if it is feasible to recruit and achieve adherence to a transfusion protocol when randomising patients to one of two haemoglobin thresholds. The study will be considered feasible and worthy of further development into a larger randomised trial if at least half of eligible patients are randomised, and if there is no evidence that the adherence to the protocol (such that transfusions are given appropriately in accordance with the randomised haemoglobin threshold policy) is lower than 70% in either arm.
The results will help design a larger multi-centre randomised, controlled trial to be conducted in the future, or may show that it is not possible to carry out such a trial.

Ethics approval required
Ethics approval required

Ethics approval(s)
Approved 24/06/2019, London – Camden and King's Cross REC (Friends House, Margaret Fell Room, 173 - 177 Euston Road, London, NW1 2BJ, United Kingdom; +44 (0)2071048238; camdenandkingscross.rec@hra.nhs.uk), ref: 19/LO/0714

Primary study design
Interventional

Study design
Interventional; Design type: Process of Care, Management of Care

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Red cell transfusion

Interventions
This is a small pilot feasibility study for a possible randomised controlled trial comparing clinical outcomes and quality of life following two different transfusion strategies in children undergoing allogeneic hematopoietic stem cell transplant (HSCT).

Children who undergo allogeneic HSCT usually receive red blood cell transfusions in the initial period after their transplant, due to reduced haemoglobin levels as a result of bone marrow suppression. In this situation, the red cell transfusions are commonly given when the child's haemoglobin falls below 70-80g/L. The evidence for this practice is based largely on clinical trials which suggest that in most situations transfusing red cells at haemoglobin thresholds of 70g/L is sufficient, with no benefit of transfusing at higher haemoglobin thresholds. However, this evidence is largely from adult trials in non-HSCT patients. Clinical studies are required to help us understand the best strategy for use of red cell transfusions in paediatric HSCT.

Patients aged between and including 1 and 17 years will participate in this study for the first 100 days after their transplant. Patients will be randomised between the two red cell transfusion strategies: transfusing at a haemoglobin threshold of < = 65 g/L (Arm A) or < = 80 g/L (Arm B).

Participants of 8 years and older will be asked to fill in short questionnaires about their quality of life at certain intervals during the period of their study. All parents will be asked to fill in an equivalent parent questionnaire.

Intervention Type
Other

Primary outcome(s)
Current primary outcome measures as of 20/01/2025:
Recruitment: The percentage of eligible children recruited and randomised into the study
Adherence: The percentage of Hb measurements where appropriate action was taken in accordance with the randomised policy

Previous primary outcome measures:
Adherence outcomes:
1. The proportion of enrolled participants for whom the transfusion policy was successfully followed
2. The proportion of red cell transfusions given in accordance with the randomisation policy where the correct dose was given
3. The mean pre-transfusion, post-transfusion and overall haemoglobin concentration (g/L) up to day 100 of HSCT and the difference between the two arms

4. The percentage of pre-transfusion haemoglobin concentrations falling at or below, or above the threshold haemoglobin of the red cell transfusion threshold assigned
5. The percentage of post-transfusion haemoglobin concentrations falling below, or at and above the target haemoglobin of the red cell transfusion threshold assigned
6. Drop out: the proportion of randomised participants who were withdrawn from the study
7. Quality of Life (QoL) questionnaire compliance: the proportion of QoL questionnaires completed at each time point

Key secondary outcome(s)

Current secondary outcome measures as of 20/01/2025:
Adherence outcomes:
1. The proportion of enrolled participants for whom the transfusion policy was successfully followed
2. The proportion of RBC transfusions given in accordance with the randomised policy where the correct dose was given
3. The mean pre-transfusion, post-transfusion and overall Hb concentration up to Day 100 of HSCT and the difference between the two arms
4. The percentage of pre-transfusion Hb concentrations falling at or below, or above the threshold Hb of the red cell transfusion threshold assigned
5. The percentage of post-transfusion Hb concentrations falling below, or at and above the target Hb of the red cell transfusion threshold assigned
6. Drop-out: the proportion of randomised participants who were withdrawn from the study
7. Quality of life (QoL) questionnaire compliance: the proportion of QoL questionnaires completed at each time point

Clinical outcomes:
1. Death: All-cause mortality at Day 100 of HSCT
2. Clinically significant bleeding (WHO Grade 3-4)
3. Red Blood Cell Exposure: The RBC transfusion volume per recipient weight (ml/Kg), number of red blood cell units administered and number of RBC transfusion episodes up to death/Day 100 of HSCT (whichever comes first). Number of patients requiring an additional transfusion within 24 hours, and reason
4. Proportion of participants experiencing thromboembolic and ischaemic events
5. Transfusion reactions
6. Grade of acute graft versus host disease and Bearman toxicity score
7. Veno-occlusive disease
8. Admission to Paediatric Intensive Care (reason and duration of stay)
9. Number of platelet transfusions
10. Health-related QoL scores

Previous secondary outcome measures:
Clinical outcomes:
1. Death: all-cause mortality at Day 100 of HSCT
2. Clinically significant bleeding (WHO Grade 3-4)
3. Red cell exposure: the red cell transfusion volume per recipient weight (ml/Kg), number of red cell units administered and number of red cell transfusion episodes up to death/Day 100 of HSCT (whichever comes first)
4. Number of patients requiring an additional transfusion within 24 hours, and reason
5. Proportion of participants experiencing thromboembolic and ischaemic events
6. Transfusion reactions
7. Grade of acute graft versus host disease and Bearman toxicity score
8. Veno-occlusive disease

9. Admission to Paediatric Intensive Care (reason and duration of stay)
10. Number of platelet transfusions
11. Health-related Quality of Life Scores.

Completion date
30/06/2023

Eligibility

Key inclusion criteria
1. Children undergoing allogeneic HSCT
2. Aged at least 1 year and under 18 years at time of consent
3. Expected to require red cell transfusions

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Child

Lower age limit
1 Years

Upper age limit
18 Years

Sex
All

Total final enrolment
34

Key exclusion criteria
1. Patients for whom the attending haematologist feels allocation to either a restrictive or liberal policy of red cell transfusion is not appropriate (e.g. acutely unwell, bleeding or 'unstable'.
2. Children undergoing HSCT for haemoglobinopathy or red cell aplasia.

Date of first enrolment
01/06/2019

Date of final enrolment
01/12/2020

Locations

Countries of recruitment
United Kingdom

England

Scotland

Study participating centre
University Hospitals Bristol NHS Foundation Trust
Marlborough Street
Bristol
United Kingdom
BS1 3NU

Study participating centre
NHS Greater Glasgow and Clyde
J B Russell House
Gartnavel Royal Hospital
1055 Great Western Road
Glasgow
United Kingdom
G12 0XH

Study participating centre
Sheffield Childrens Hospital
Western Bank
Sheffield
United Kingdom
S10 2TH

Study participating centre
Manchester University NHS Foundation Trust
Cobbett House
Oxford Road
Manchester
United Kingdom
M13 9WL

Sponsor information

Organisation
NHS Blood and Transplant

ROR
https://ror.org/0227qpa16

Funder(s)

Funder type
Government

Funder Name
NHS Blood and Transplant; Grant Codes: TF070

Results and Publications

Individual participant data (IPD) sharing plan
The datasets generated during and/or analysed during the current study are/will be available upon request from NHSBT Clinical Trials Unit (CTU@nhsbt.nhs.uk).

IPD sharing plan summary
Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article		21/04/2025	15/07/2025	Yes	No
HRA research summary			28/06/2023	No	No