REDRESS

King's College Hospital NHS Foundation Trust

King's College London

UKCRC Registered Clinical Trials Units

Study Introduction Information Slides

RElated haplo-DonoR haematopoietic stEm cell transplantation for adults with Severe Sickle cell disease (REDRESS)

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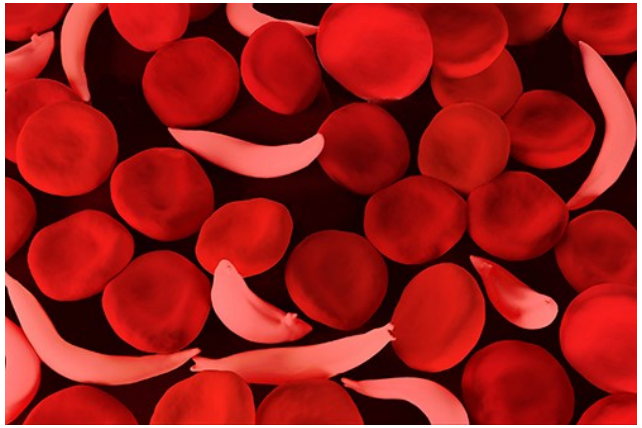
Background & Rationale

- Sickle Cell Disease (SCD)
  - Inherited condition caused by abnormality of haemoglobin.
  - Shorter Life expectancy, sickle crisis, health issues, impaired quality of life
  - Require lifelong healthcare and access to the NHS services, especially for those with severe sickle

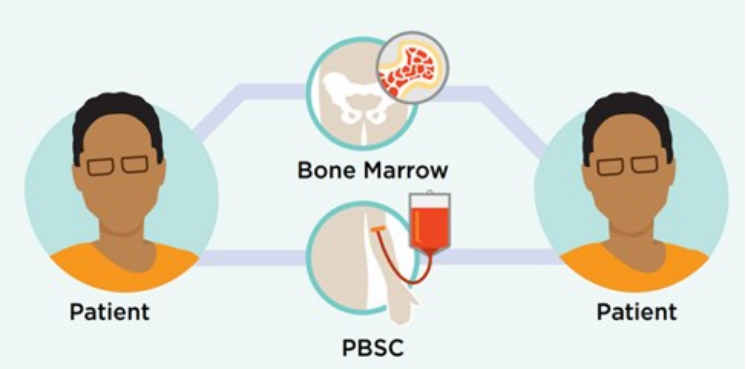


- Available licensed therapies in UK
  - Blood transfusions, hydroxycarbamide
  - Not curative.

- Stem Cell Transplantation (SCT)
  - Chemotherapy, immunosuppression, stem cell transplanted from **FULLY** matched sibling donors.
  - Established globally to treat and cure those with severe disease
  - Cure rates are **HIGH** (85-87%)

Background & Rationale continued

- Haploidentical stem cell transplant (*half-matched*)
  - **70%** of 'in need' SCD population do not have a suitable sibling donor
  - Haploidentical (half-matched) family donors almost universally available
  - Haplo transplant routinely delivered for U18s in UK
  - New protocols now allow haplo transplant to be used in over 18s
  - **BUT… We need more evidence!**

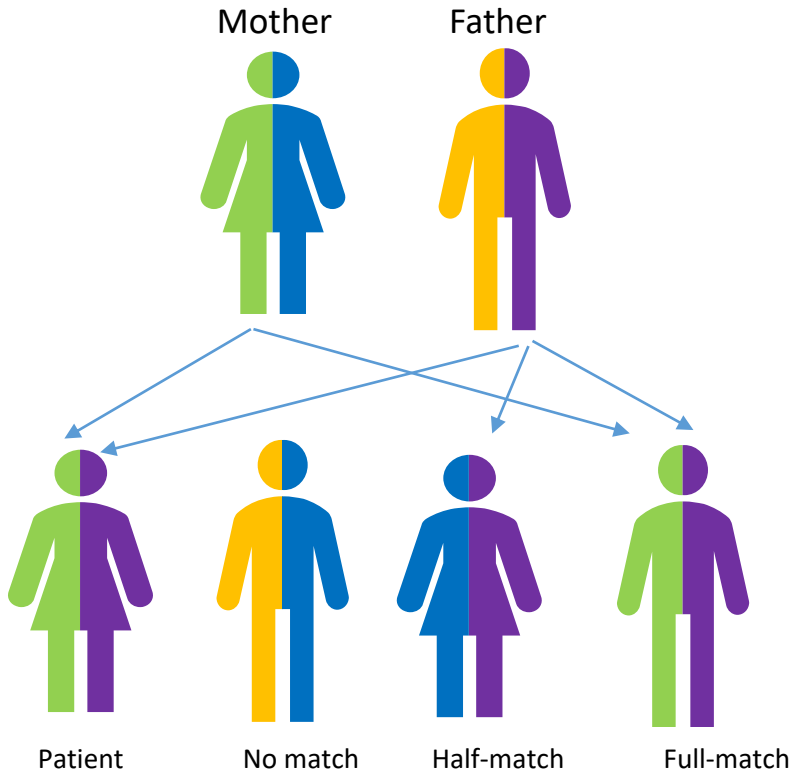


- Cost-effectiveness of haplo transplant v standard care
  - Haplo transplant is expensive but current cost to NHS is potentially more expensive
  - Potential huge cost savings

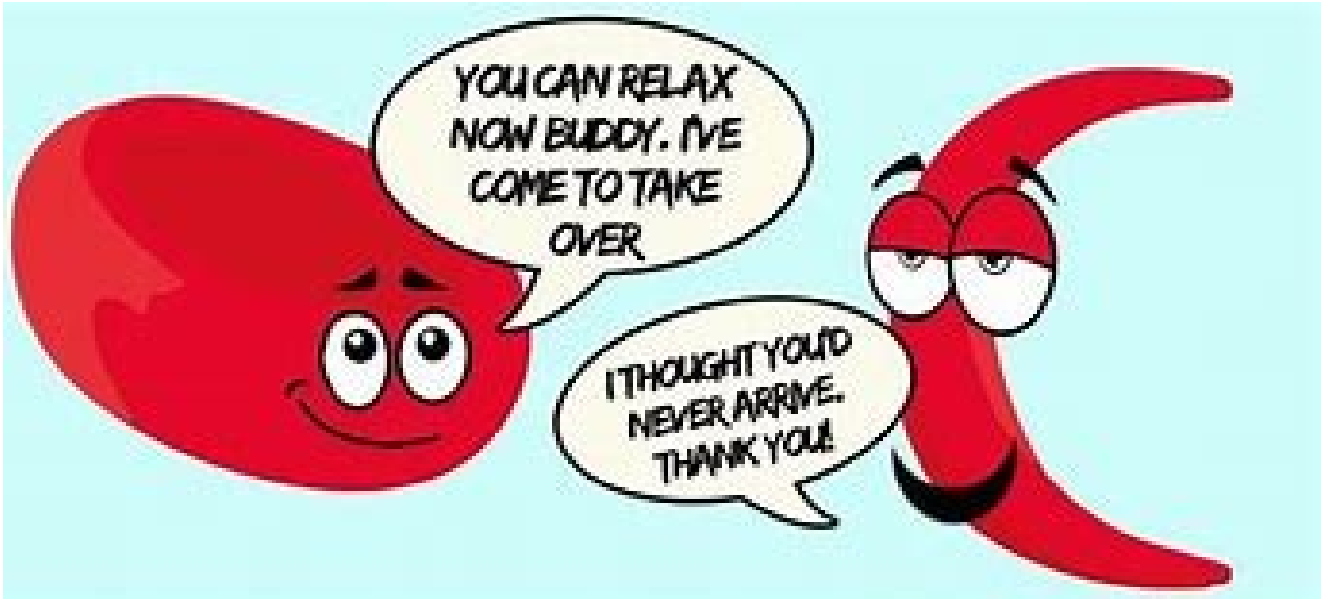
However most people will have a haplo donor

Donor matching

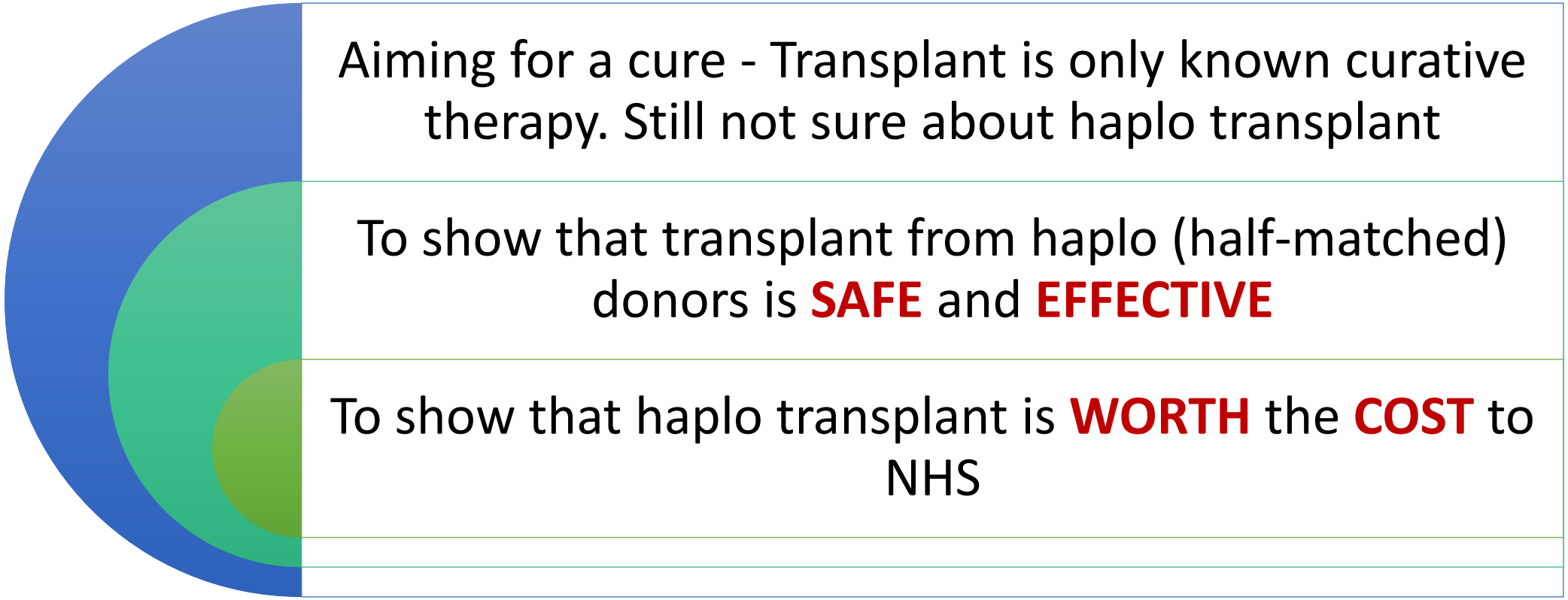
human leukocyte antigen-HLA system
(important immune cell proteins)



- HALF MATCHED DONOR
- CAN BE PARENT, CHILD OR SIBLING
- CAN HAVE SICKLE CELL TRAIT



Aims of REDRESS study



If haplo transplant shown to effective, safe and cost-worthy, it would give those most in need with a donor option, having a major impact on the quality and length of life for those with sickle cell.

Inclusion Criteria

a) Adult patients age ≥ 18 years

b) Confirmed haploidentical donor

c) Severe SCD who are at high risk for other complications and death. Severe SCD includes:

   i. Strokes
   ii. Chest pains
   iii. Regular sickle crisis
   iv. Regular transfusions
   v. Patients needing treatment of SCD complications who cannot tolerate either hydroxycarbamide or transfusions.
   vi. Organ damage.

d) Patients must be fit enough to have haplo transplant

e) Written informed consent.

Exclusion Criteria

a) Fully matched sibling donor.

b) Previous bone marrow transplant.

c) Pregnancy or breast feeding.

d) Participants able to conceive a child that are unprepared to use effective contraception.

e) Clinically significant donor specific HLA antibodies.

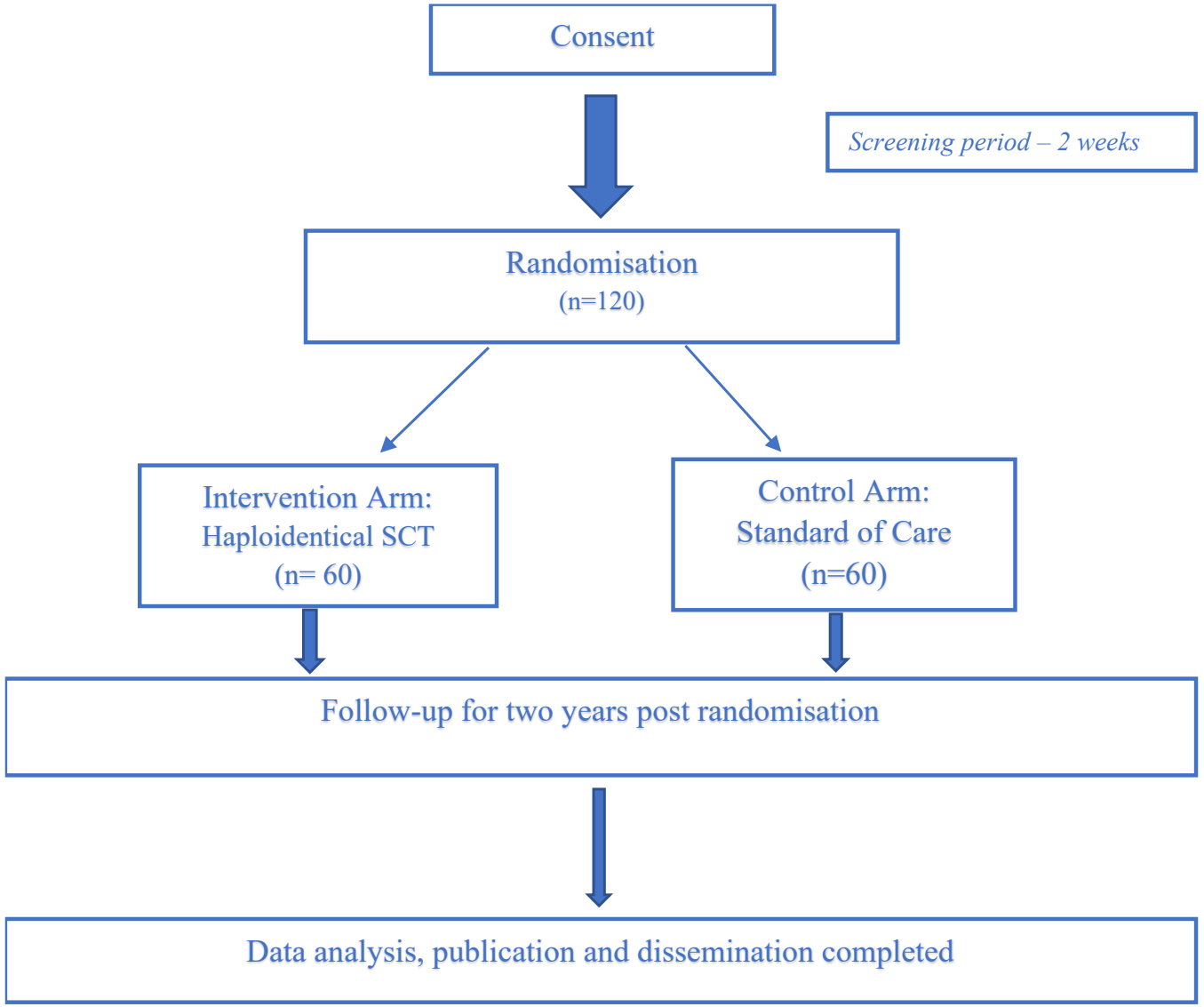
f) HIV infection or active Hepatitis B or C.

g) Uncontrolled infection including bacterial, fungal and viral.

h) Participation in another interventional trial in the last three months.

i) Pre-existing condition deemed to significantly increase the risk of haplo transplant by the local Principal Investigator.

Study Design



Donor Eligibility

- **HAPLOIDENTICAL matched family donor**
- **Donor must be identified PRIOR to enrolment of transplant candidate**
- **Donor must be assessed as FIT to donate stem cells**

Study Status

- Regulatory Approvals
  - Ethics Committee & Regulatory approval: **November 2022**
- Study is **Open**
  - First site = King's College Hospital, London
  - Plan to open approx. 10 sites
  - Coming to a centre near **YOU!**
- Public Launch Event – 20 May 2023
  - Attended by patients, doctors, researchers, supporters, public
  - High interest and appetite for new treatments amongst community.


Curative therapies
• <15% SCD have matched sib
• (brother or sister born of same mum and dad)
• BUT 90% will have a Haplo donor
After matched sibling transplant
95 of 100 patients survive procedure
> 85 of 100 cured
REDRESS
REDRESSing the balance of healthcare for patients with sickle cell
Learn more: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Thank you for your time!

Any questions?