Allogeneic stem cell transplantation for sickle cell disease in Australia and New Zealand

Outcomes, unmet demand and the arrival of gene therapy

Nada Hamad on behalf of the ANZTCT Registry and co-investigators · ASM 2026

ANZTCT ASM 2026

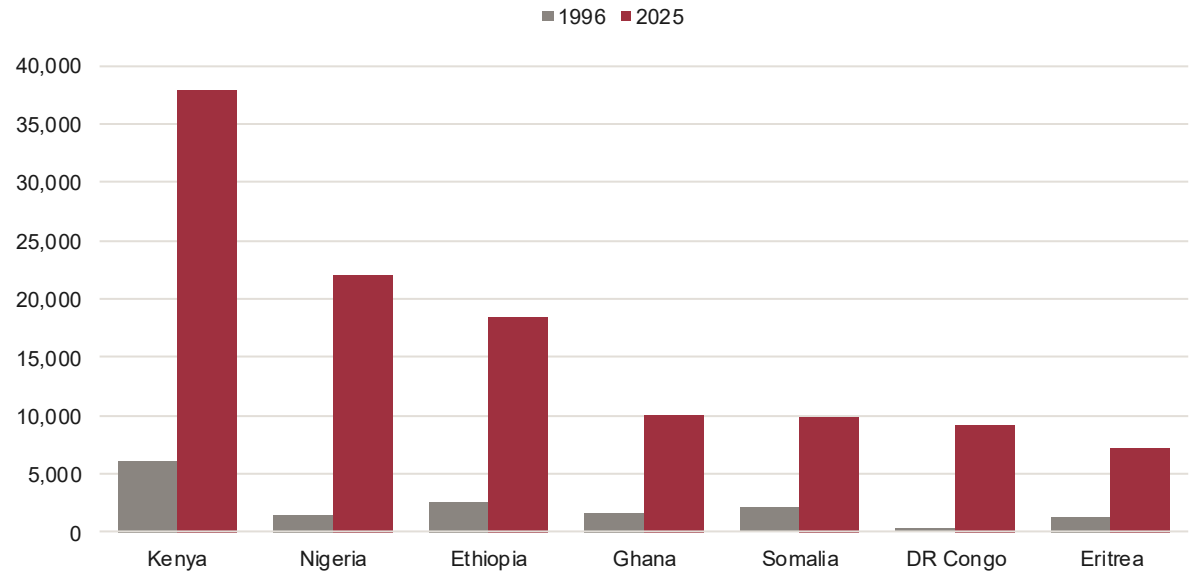
Art by Lola-Anne Davis

Sickle cell disease is a migration story before it is a haematology story.

It arrived here with people.

Our referral pathways were built for a population that no longer exists.

The population has transformed



Australian residents by country of birth, 1996 and 2025.
Source: ABS, Australia's population by country of birth, cat. 3409.0, June 2025.

Over the same period

Nigeria ×15
1,460 to 22,020

DR Congo ×29
320 to 9,230

Iraq ×7
16,400 to 111,880

Syria ×6
6,620 to 38,800

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Aim and method

- **Registry analysis**
- All allo-HSCT for SCD recorded in the ANZTCT registry, January 2011 to June 2026
- Retrospective analysis
- Overall and event free survival, engraftment, graft failure, acute and chronic GVHD, transplant related mortality

- **National health services study**
- Annualised data from paediatric centres across Australia and New Zealand
- SCD prevalence, patients on chronic transfusion or red cell exchange, and those proceeding to allo-HSCT
- Purpose: define the population eligible for transplantation and for gene therapy

Twenty one transplants in fifteen years

21

patients underwent allo-HSCT for SCD

20

paediatric, and one adult

11

years median age, range 3 to 25

1 → 4

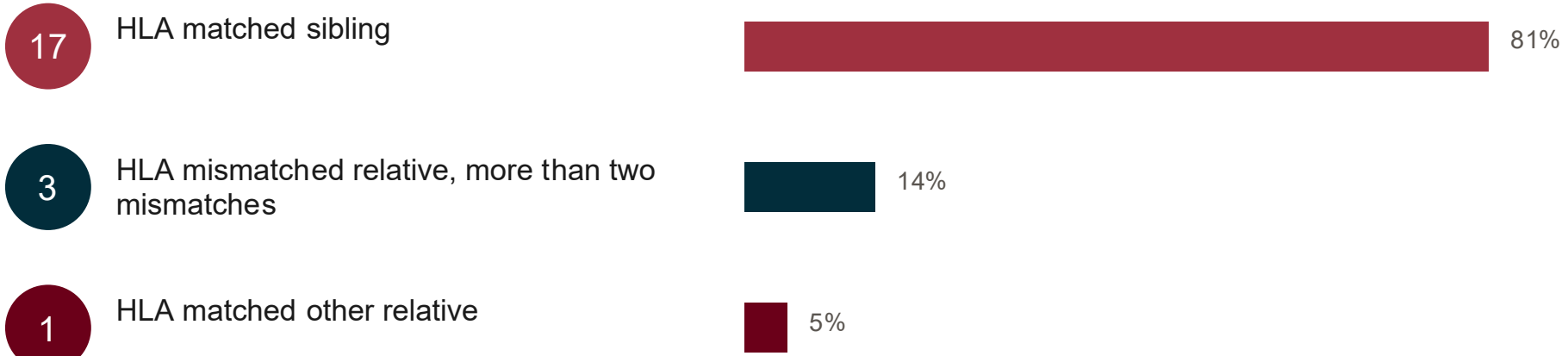
per year, 2011 to 2024

Annual numbers are rising. The population driving them is rising faster.

Source: ANZTCT registry, January 2011 to June 2026.

Donor source

17	HLA matched sibling	81%
3	HLA mismatched relative, more than two mismatches	14%
1	HLA matched other relative	5%



Overwhelmingly matched sibling donors.

That is a selection effect, not a clinical preference, and it quietly excludes every child without one.

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Two transplant related deaths

Day 160

Graft versus host disease

A 17 year old patient.

Day 315

EBV related lymphoproliferative disease

With rasburicase induced methaemoglobinaemia.

Two deaths in twenty one patients, both transplant related.

Survival in this series is excellent.

The risk of transplant is now lower than the cumulative risk of a lifetime of untreated sickle cell disease.

Curative. Safe enough.
And almost nobody is getting it.

Twenty one patients in fifteen years, across two countries.

The question is not whether the treatment works.

The question is who is never referred.

We know the numerator. Not the denominator.

8,485

estimated to be living with SCD in Australia, 2021

357

on the Australian Haemoglobinopathy Registry, 12 sites

21

transplanted in Australia and New Zealand since 2011

The registry sees about 4% of the modelled prevalence. We transplant about 0.25% of it.

Different sources, timeframes and definitions: modelled prevalence 2021, registry snapshot 2021 to 2022, transplants cumulative 2011 to 2026. Bars to scale.

Sources: Nwokeocha et al., Intern Med J 2025;55:1251. Nelson et al., Intern Med J 2024;54:764. ANZTCT registry.

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Two things are about to change the numbers

Newborn screening is here

Health Ministers agreed to add SCD to the national newborn bloodspot panel on 25 September 2024. Victoria began screening on 11 February 2026. Targeted testing was finding fewer than half of cases before symptoms.

Gene therapy is licensed

Autologous gene therapy removes the donor problem entirely, and replaces it with a cost and capacity problem. It will be rationed. Rationing without a denominator is rationing by accident.

Screening will find them. We have not yet decided where to send them.

Sources: Department of Health, Disability and Ageing newborn bloodspot screening panel. MSAC Application 1737.1, November 2023.

An equity problem, not a capacity problem

The barrier is not the transplant

Outcomes are good. Beds exist. The failure is upstream, in diagnosis, referral and who is thought of as a transplant candidate.

The affected population is the least served

Recent migrants and humanitarian entrants, often with limited English, no family history on file and no continuity of care.

Matched siblings are unevenly distributed

Family structure after displacement is not a clinical variable, but here it decides who is eligible.

You cannot plan for a population you have not counted

Culturally safe models of care and service design both depend on knowing who is out there.

Conclusion

- Allo-HSCT is curative for SCD with excellent survival, and is grossly underused
- Demand will grow with demographic change and with newborn screening now underway
- Licensed gene therapy expands curative options and sharpens the need for planned, equitable access
- Defining the national denominator is the critical next step, to quantify unmet need and to guide policy, culturally safe models of care and service development

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