

Overview status of Personalized Medicine in Tanzania

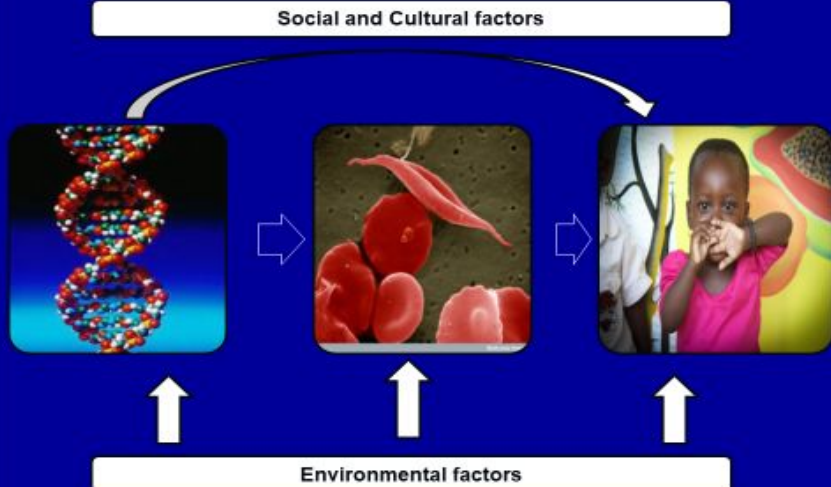
Dr. Agnes Jonathan, Dr. Emmanuel Balandya, Dr. Siana Nkya, Florence Urio, Daima Bukini, Julie Makani on behalf of Sickle CHARTA, SickleInAfrica, and Sickle Cell Programme, MUHAS

13 July 2022



Sickle Cell Disease: A Model for Personalized Medicine

Sickle Cell Disease



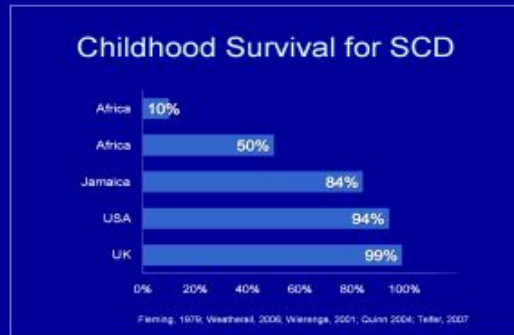
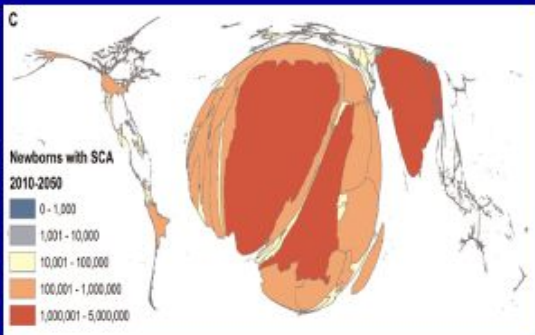
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Sickle Cell Disease – 1st “Genetic” Disease



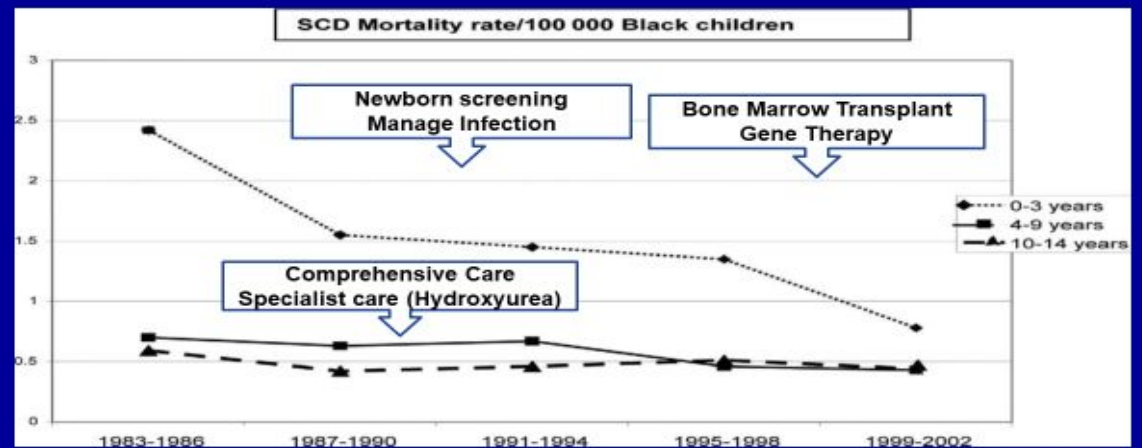
Pauling 1949

Sickle Cell Disease: Unmet Medical Need



Piel 2013; Fleming 1979, Wierenga 2001, Quinn 2004, Weatherall 2006, Telfer 2007, Makani 2011

Sickle Cell Disease: Public Health



Yanni 2009

Treatment Options for Sickle Cell Disease

Current treatments

Future treatments

Exchange Blood Transfusion*

Drug treatment

Aim to allow more cells to transit the microcirculation before sickling

Allogeneic transplant

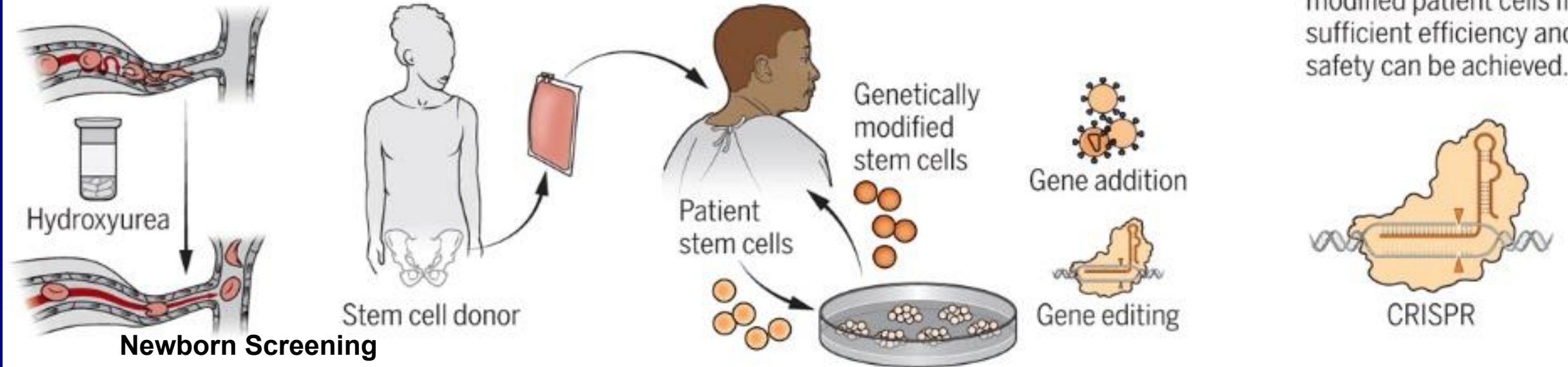
An established curative strategy using bone marrow stem cells from a donor without SCD

Ex vivo gene therapy

The patient's bone marrow cells are modified by adding a β -globin gene, using a retroviral vector or with gene editing, to reactivate fetal hemoglobin (HbF) or correct the disease mutation.

In vivo gene therapy

Direct gene editing in patients could circumvent the need for transplantation of modified patient cells if sufficient efficiency and safety can be achieved.

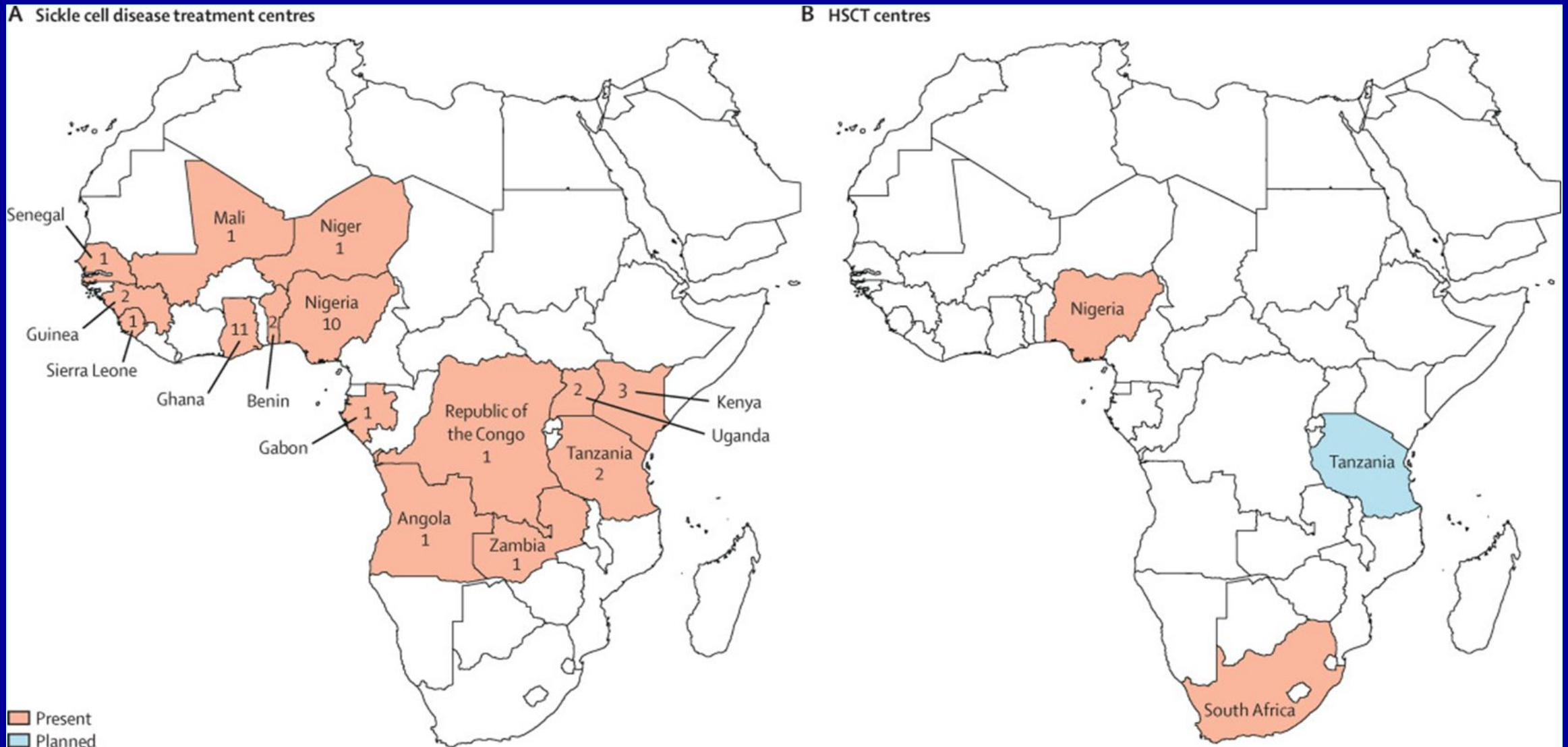


2020*

2025

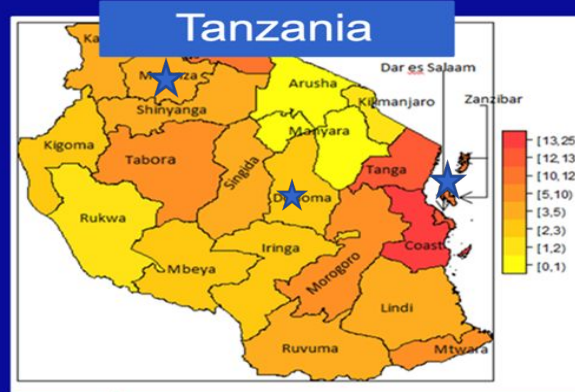
2030

Personalized Medicine for SCD in Africa



Personalized Medicine for SCD in Tanzania

Muhimbili National Hospital



- Tanzania
- Population: 60 Million
- Annual SCD Births: 11,000
- SCD in Population: 200,000

Aga Khan Hospital



Bejamini Mkapa Hospital



BMH yaanza kubadilisha chembe nyekundu za damu kwa wenye seli munda

@bmhdodoma

JKCI



Bugando Medical Centre



Personalized Medicine for SCD: Resources

Transplant unit



- Experience with renal transplant at MNH
- Capacity will benefit patients with other haematologic diseases (e.g; acute leukemia)
- Experience gained in BMT will facilitate gene therapy
- Facilities in place – MNH, BMC, BMH, Aga Khan (ICU, isolation rooms etc.)
- MUHAS – Upanga and Mloganzila

Gene & Cell Laboratory in MUHAS



HLA Typing

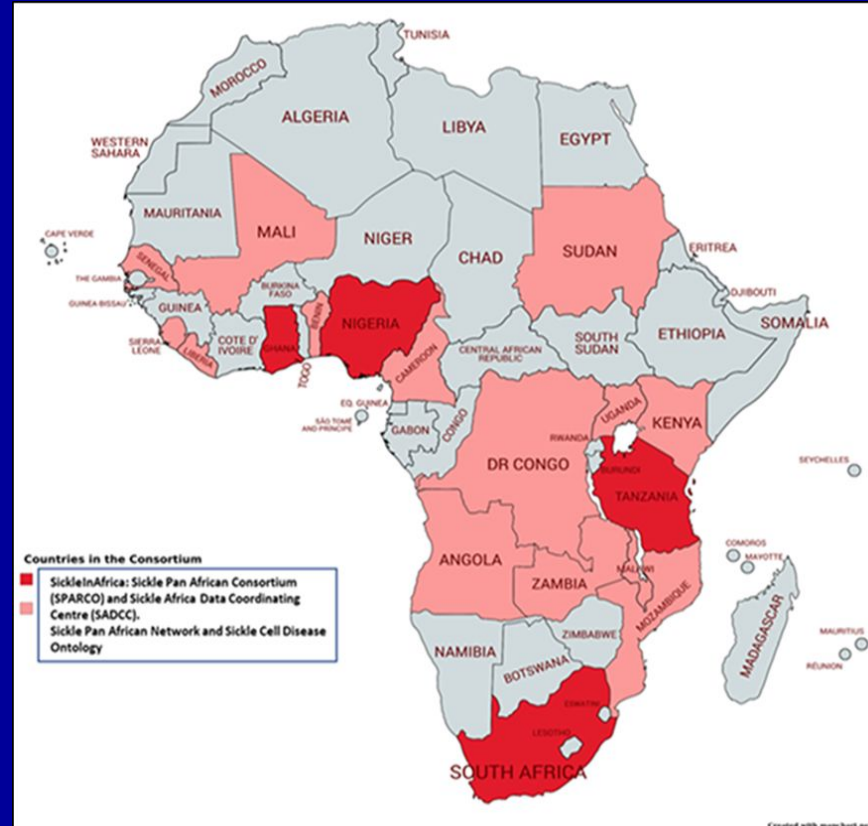


Personalized Medicine for SCD: Health

Sickle Cell Clinics



SCD Registry: 13,000 – 34,000



Gene and Cell Therapy



Blood Transfusion



Areas to Address

Should Africa prioritize Comprehensive Care or Research for Cure?

Can Curative Therapies be provided in Africa?
Where? Who? Cost?

Resources are limited.
Why don't you focus on interventions that will help many?

Transplant or Gene Therapy?

Which Gene Therapy approach?

Is the patient community ready?

Are there regulatory frameworks?

Why is Equal Access to New & Curative Therapies important in Africa?

Ethics, Equity, Expertise, Experience

Ghana, Nigeria, Tanzania; Mali, Uganda, Zambia/Zimbabwe (7/54)
Global SCD: 5,000,000
SCD in Africa: 3,500,000 (75%)
SickleInAfrica 2020. Lancet Haematology

Personalized Medicine for SCD: Healthcare at all levels

Diagnosis



Comprehensive Care



Counselling



Disease- Modifying Medicines



Gene and Cell Therapy



Patient Centered Approach



Personalized Medicine for SCD: Personnel

Personnel

Laboratory Technical Team



Siana Nkya
Lead, HbF genomics



Florence Urio
HbF genomics



Mohammed Zahir
CD34



Aisha Rifai
HLA Typing



Heavenlight Christopher
Molecular Diagnostics



Fridah Kaywanga
CD34

Clinical Technical Team



Dr. Grace Moshi
Lead, Haematologist



Prof. Julie Makani
Haematologist



Prof. Lucio Luzzatto
Haematologist



Dr. Vivien Sheehan
Haematologist



Dr. Fredrick Luoga
Exchange Transfusion



Dr. Deos Maingu
Pre-transplant Eligibility (HSCT)

ELSI & Patient Engagement Technical Team



Daima Bukini
Lead, Gene Ethics



Jennifer Mashaka
Research Fellow



Arafa Salim
Patient Advocate Lead



Bora Hilary
Patient Advocate Lead



Neema Mohamed
Patient Advocate Lead



Ruth Lwakatare
Patient Engagements Support

Training

- Training in Tanzania
MUHAS: UG (MD, BMLS) and PG (MMed/MSc/PhD)
Short/long-term curricula on BMT being developed.
- Training Outside Tanzania
Long-term
China: 4 trainees in BT/BMT (2018)
Short term
France, Italy, India, USA, UK
- Continuing Education and Professional Development

Personalized Medicine for SCD: Partnerships

Government



Health Agencies - WHO



Financing Agencies



Industry



Philanthropy



Community



Thank You! Asante!

Individuals, Community, Healthcare Providers, Researchers, Government, Industry, Non-government Organizations, Funders

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