



# **ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION IN CHILDREN WITH SICKLE CELL DISEASE: PRELIMINARY RESULTS OF THE FIRST PATIENTS TRANSPLANTED IN TANZANIA**

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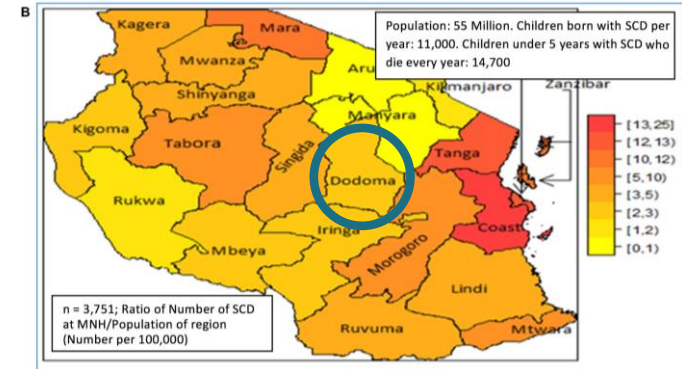
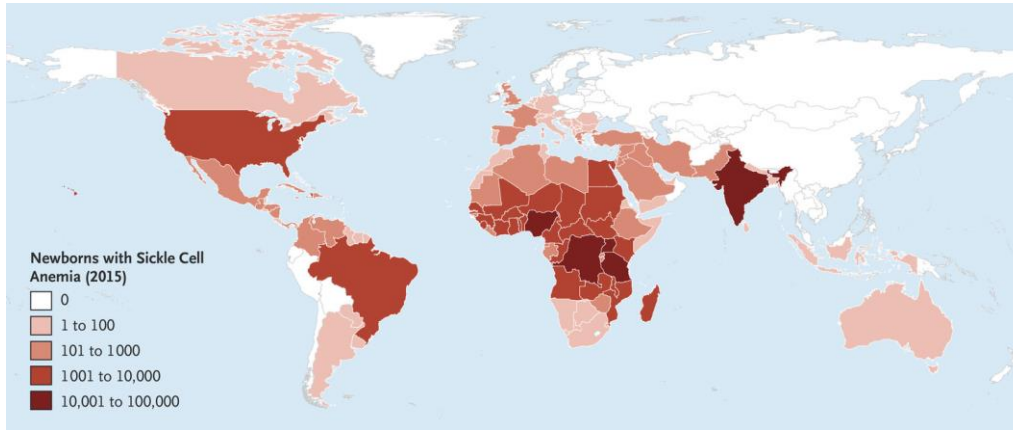
IRCCS San Raffaele Scientific Institute, Milano, Italy

# Disclosure statement

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Nothing to disclose

# SCD in Tanzania

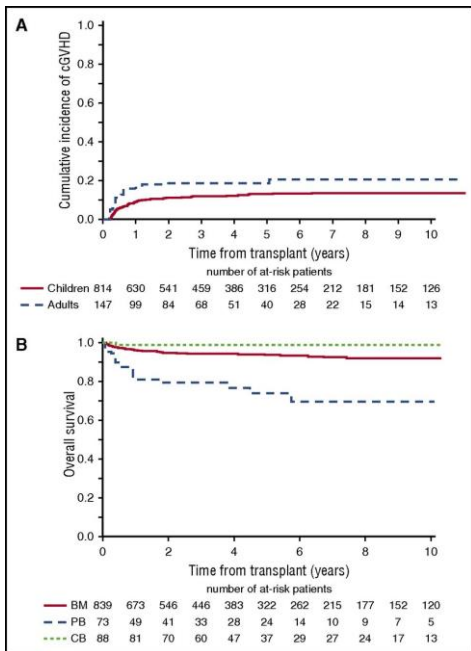


- Between 2010 and 2050, 14 million children will be born with SCD worldwide, with 84% of these from Africa.
- Survival in Africa is as low as 10%.
- Mortality in Tanzania reported at 1.9 (95% confidence interval, 1.5–2.9) per 100 person-years observation (PYO).
- The mortality rate is highest in children under the age of 5.
- In Tanzania at least 14,700 deaths among children under the age of 5 every year could be attributed to SCD.

FB. Piel et al, N Engl J Med 2017

J. Makani, Hematology/Oncology and Stem Cell Therapy, 2020

# SCD: international survey of results of HLA-identical sibling HSCT

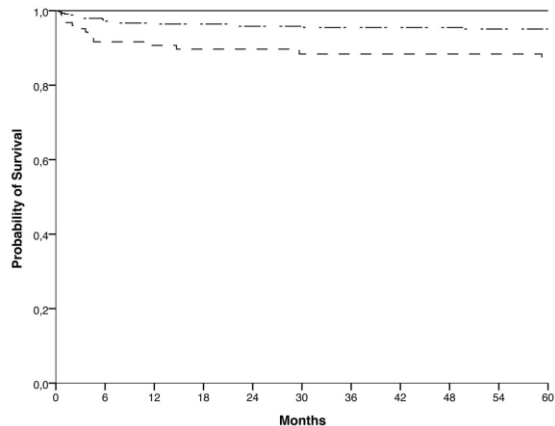


1986-2013 → 1000  
HLA-identical sibling  
HSCT at 106 centers  
in 23 countries.

Median follow-up 55  
months (3-325).

439 in USA  
513 in Europe  
48 in non-European  
countries.

4-year OS



1986-2017 → 736 HLA-identical sibling HSCT  
(EBMT/Eurocord)  
0-5 years: group 1 (n=175)  
6-15 years: group 2 (n= 436)  
>15 years: group 3 (n=125)

# The way to HSCT



2018  
Tanzania development program to establish HSCT

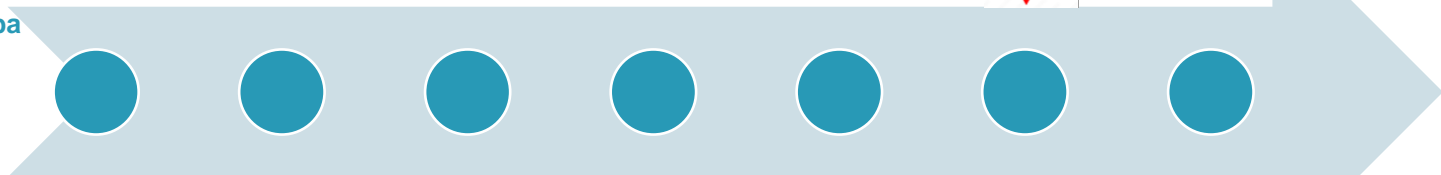
- 50 videoconference lectures by international HSCT experts
- 4 nurses, 3 doctors and 2 laboratory scientists took part in internship in Italy

2020

2022



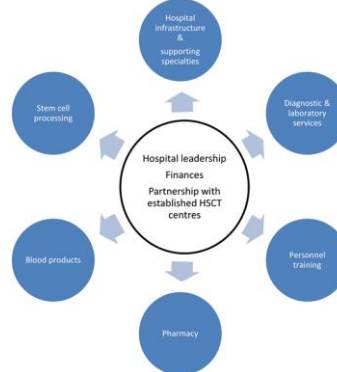
2024  
January  
HSCT going on with 10 experts for 2 months



2019  
MoU with Association HELP3-ODV ([www.help3.it](http://www.help3.it))



2021

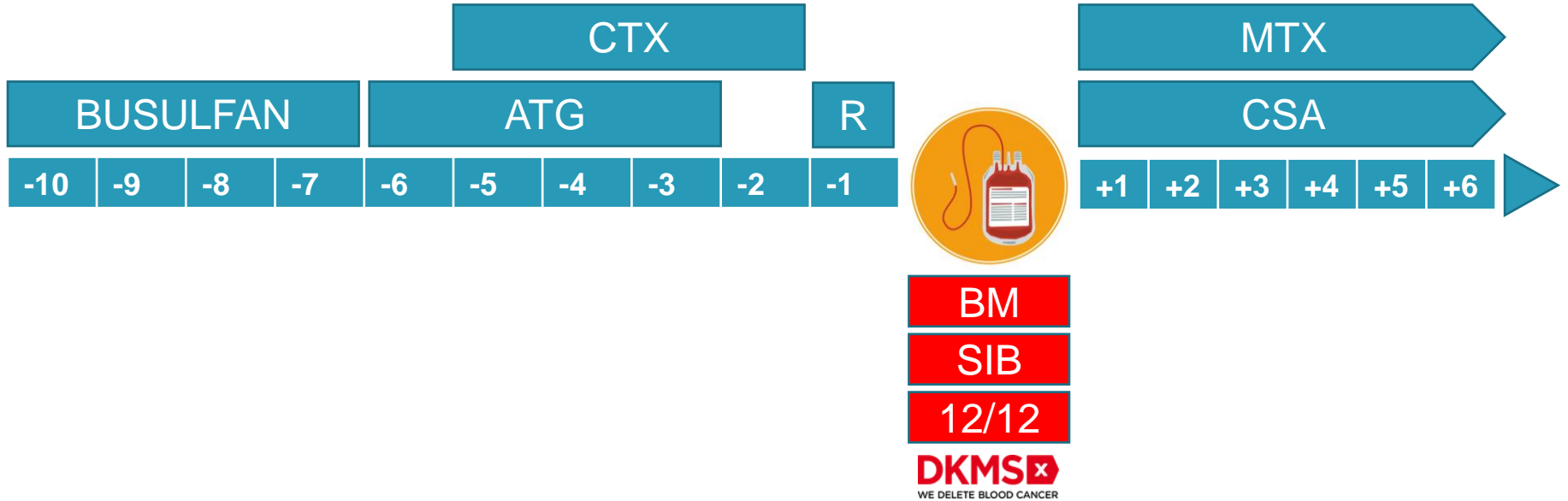


2023

January  
HSCT program started with 12 Italian experts in Tanzania for 4 months

9 patients transplanted

# Methods



- Levetiracetam
- Antimicrobial prophylaxis
- Normal oral nutrition

# Results



January 2023 – April 2024: 9 symptomatic SCD patients transplanted (8 patients engrafted, 1 patient ongoing).



	All patients N=8
Median follow-up	224 days (57-421)
Median Age at HSCT (range)	8 years (4-11)
Sex (Female/Male)	2F/6M
Preparatory Automated Red Cell Exchange	8/8
MAC+ATG	8/8
CSA+MTX	8/8
ABO match	8/8
Median TNC x 10 <sup>8</sup> /Kg (range)	4,7 (2,6-8,9)
Donor Trait	5/8
Median Donor Age (range)	5 (1-8)

# Results: early outcomes



	All patients N=8	
ANC engraftment	8/8	+28 (19-41)
PLT engraftment	8/8	+22 (15-35)
Chemo Toxicity	8/8	
Nausea/Vomiting	1/8	Max grade 1-2 CTCAEv5
Mucositis	8/8	Max grade 1-2 CTCAEv5
Blood Hypertension	2/8	Max grade 1-2 CTCAEv5
Febrile Neutropenia	8/8	
Viral	1/8	1 CMV
Bacterial	2/8	1 Coagulase-negative Staphylococci 1 Gram+rods
Fungal	0/8	4 Galattomannan positive pts without other signs of IFD → no treatment
Parasyte	1/8	P. Falciparum
aGVHD	1/8	Grade II Skin aGVHD on +21 treated with MPD and MMF → complete resolution



# Results: late outcomes



	All patients N=8	
cGVHD	0/8	
Late Infections	2/8	
Viral	1/8	Oral HPV → cryotherapy
Bacterial	0/8	
Fungal	1/8	Probable IFD → Voriconazole
Parasyte	0/8	
IS Stop	4/8	+292 (265-300)
Back to School	3/8	
SCD Events	0/8	
Blood Transfusions	0/8	
Full chimerism (HbS Level)	8/8	

# Conclusions



First 9 allogeneic sibling HSCT performed in Tanzania.  
HSCT is feasible in LMICs with comprehensive adjustments on infrastructures and human resources. To note that *Tanzanian staff* never had past experience in HSCT procedures

- The first 8 patients are now asymptomatic with full chimerism.
- The first 4 patients stopped IS.
- The first 3 patients are back to school.
- No major safety concern.

Patients N.4,5,6,9 have been transplanted without the Italian staff at BMH.  
Weekly online clinical-logistical round started in March 2023 and are ongoing.

Crucial to maintain a high level of standardization (SOPs) on HSCT procedures.  
Supply chain of drugs and consumables remain a major issue.  
An interaction among all the hospital services is mandatory.

# Future Directions

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Tanzanian government has deliberated to support BMT at BMH Hospital.

It'll be fundamental to ensure sustainability of the service considering the immense need of HSCT.

Maintaining the cost affordable and creating a reliable supply chain of drugs and consumables will be crucial (Blood Bank particularly relevant).

An ad hoc analysis is ongoing to evaluate the cost-effectiveness of HSCT at BMH, comparing HSCT cost to standard of care of SCD patients (considering direct/indirect costs).

Continuous training for health professionals is ongoing and needs to be implemented.

Expand HSCT to ABO mismatch host/donor.

Expand HSCT to other disorders.

Collaborate with other Tanzanian Hospitals to create a BMT Network.



# 50<sup>th</sup> Annual Meeting

**Glasgow**

**14-17 April 2024**

*Celebrating 50 Years of Community*

**#EBMT24**

David Mashala  
Stella Malangahe  
Shakilu Jumanne  
Merlikiad Mhozya  
Paola Erminia Ronchi  
Alessia Orsini  
Francis Ngiliule  
Zimbwe Bakari  
Alen Patrick Magoha  
Anna Carmagnola  
Musa Dubali  
Nunzia Manna  
Pietro Pioltelli  
Andrea Biondi  
Fabio Ciceri  
Alphonse Chandika  
Cornelio Uderzo



**Children and their families**  
All nurses, doctors and  
people involved.



**Thanks!**



**Hospitali ya Benjamin Mkapa**



**Pediatric Hematology Oncology O**