Clinical and immunological outcome after paediatric stem cell transplantation in inborn errors of immunity

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Curriculum vitae

Su Han Lum was born on July 19th 1978 in Kuala Lumpur, Malaysia. Her fascination with paediatric haematology and haematopoietic cell transplantation began during her undergraduate days. She studied medicine (1999-2004), received her paediatric training (2008-2012) and completed her fellowship in paediatric haematology and bone marrow transplantation (2013-2015) at the University of Malaya, Kuala Lumpur, Malaysia. She underwent further training in haematopoietic stem cell transplantation and cellular therapy in Manchester and Newcastle from 2015 to 2019. She completed the Global Clinical Scholars Research Training Program (Harvard Medical School, 2016-2017), the Rare Diseases Research Training Program (NIH Rare Diseases Clinical Research Network, 2017-2018) and the Clinical Research Training in Haematology (European Haematology Association, 2019-2020). She is currently working a consultant paediatric haematologist at Great North Children's Hospital, Newcastle upon Tyne, United Kingdom

Her interests are in haematopoietic cell transplantation and cellular therapy for children with rare diseases, especially precise graft prescription and adoptive immunotherapy, and late effects.

She has been actively pursuing her passion for statistics and data science since she was a medical student. She is currently studying Master of Medical Statistics (Oxford).

Her long-term vision is to be an academic paediatric transplanter who improves the transplant outcomes and long-term health status of children with rare diseases, through clinical trials and multicentre collaborative studies.

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Abbreviations

AD	autoimmune disease
aGvHD	acute graft versus host disease
AIC	autoimmune cytopenia
AIHA	autoimmune haemolytic anaemia
AIN	autoimmune neutropenia
ALL	acute lymphoblastic leukaemia
ALPS	autoimmune lymphoproliferative syndrome
AML	acute myeloid leukaemia
APC	antigen presenting cells
ATG	anti-thymocyte antiglobulin
AUC	area under the curve
BAL	bronchoalveolar lavage
BM	bone marrow
BMT	bone marrow transplantation
B-NHL	B-cell-Non-Hodgkin-Lymphoma
Bu	busulfan
CB	cord blood
CGD	chronic granulomatous disease
cGvHD	chronic graft versus host disease
CI	confidence interval
CID	combined immune deficiency
CIN	cumulative incidence
CMV	cytomegalovirus
CSA	ciclosporin
CVA	cardiovascular accident
Cy	cyclophosphamide
DAT	direct anti-globulin test
DFS	disease-free survival
EBMT	European Blood and Marrow Transplantation
EBV	Epstein Barr virus
ECP	extracorporeal photopheresis
EFS	event-free survival
FDA	Food and Drug administration
Flu	fludarabine
FOXP3	Forkhead-Box-Protein 3
GvHD	graft-versus-host disease
GvL	graft-versus-leukaemia
GOF	gain of function
GvHD	graft-versus-host disease
HCT	haemopoietic cell transplantation
HID	haploidentical donor
HLA	human leucocyte antigen

HLH	haemophagocytic lymphohistiocytosis
HSCs	haematopoietic stem cells
IEI	inborn errors of immunity
IPEX	immune dysregulation, polyendocrinopathy, enteropathy, X-linked syndrome
ITP	Immune cytopenia
IVIg	intravenous immunoglobulin
IST	Immunosuppressive therapy
IUIS	International Union of Immunological Societies
JIA	juvenile idiopathic arthritis
KGF	keratinocyte growth factors
LPD	lymphoproliferative disease
LRBA	LPS-responsive beige-like anchor protein
MAC	myeloablative conditioning regimen
MAS	macrophage activation syndrome
MEC	muco-epidermoid carcinoma
MD	matched donor
MDS	myelodysplastic disorder
MHC	major histocompatibility complex
MIC	minimal intensity conditioning
Melph	melphalan
MFD	matched family donor
MMD	mismatched donor
MMF	mycophenolate mofetil
MMFD	mismatched family donor
MPT	malignancy post-transplant
MUD	matched unrelated donor
MTX	methotrexate
NK	natural killer cells
OD	odd ratio
OS	overall survival
PBSC	peripheral blood stem cell
PCP	Pneumocystis jiroveci
PCR	polymerase chain reaction
PK	pharmacokinetic
PTCy	post-transplantation cyclophosphamide
PTLD	post-transplant lymphoproliferative disease
PID	primary immunodeficiency
RAG	recombinase activating genes
RCA	red cell aplasia
RELP	restriction fragment length polymorphism analysis
RIC	reduced intensity conditioning regimen
RIT	radioimmunotherapy

RTC	reduced toxicity conditioning regimen
SAA	severe aplastic anaemia
SCID	severe combined immunodeficiency
SHR	subdistribution hazard ratio
SLE	systemic lupus erythematous
STR	short tandem repeat
TBI	total body irradiation
TBII	thyroid binding inhibitory immunoglobulin
Thio	thiotepa
TMA	transplant associated microangiopathy
TNC	total nucleated cell dose
Treo	treosulfan
TRA	thyroglobulin antibody
TRM	transplant-related mortality
UD	unrelated donor
US	United States
VNTR	variable number of tandem repeats
VOD	veno-occlusive disease
WAS	Wiskott Aldrich syndrome
WHO	World Health Organization
XIAP	X-linked inhibitor of apoptosis protein deficiency