Workshop of the WBMT in collaboration with WHO

Patient Selection

Which patients can best be served by a program

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Factors which influence patients selection toward performing HSCT

1. Few therapeutic modalities for that disease entity exist other than HSCT (from APBMT and JSHCT survey).

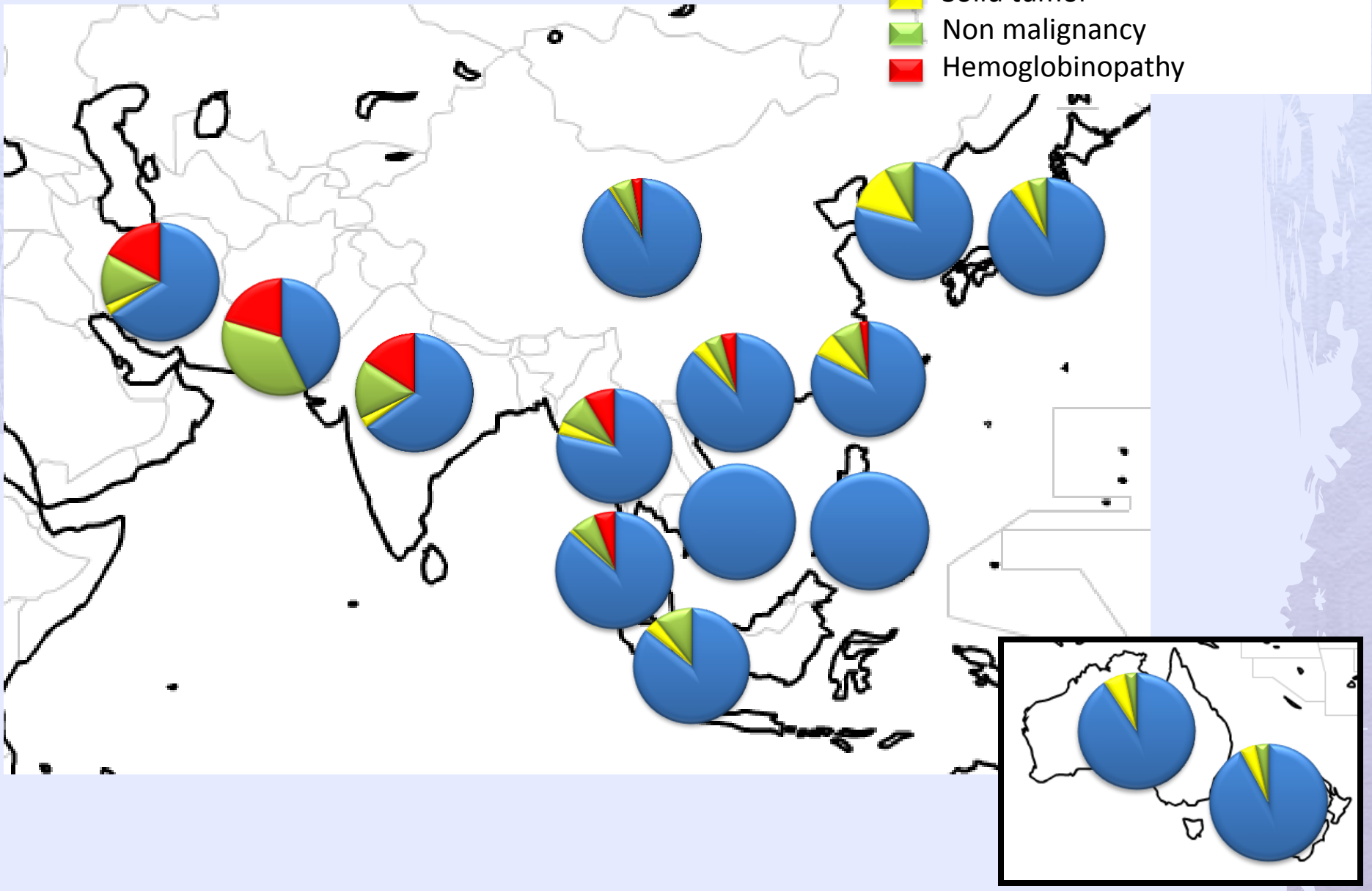
2. Few therapeutic modalities for that disease status exist other than HSCT ("definite" and " in routine use for selected patients" in the guideline).

3. Reasonably improved outcome in that disease entity/status can be expected by HSCT ("to be undertaken in approved clinical research" in the guideline).

HSCT to hemoglobinopathy, which is the disease with few curative therapeutic modalities, is dominant especially in southwest and south Asian emerging countries in APBMT where the incidence of that disease is high. These countries are also so called emerging countries where the infrastructure to cover all the demands of HSCT has not been established. As the results, HSCT teams select patients with hemoglobinopathy as one of the first candidates for HSCT. The survey results in emerging countries suggests that hereditary diseases usually occurred at young people are the first priority for allogeneic HSCT.

1. FEW THERAPEUTIC MODALITIES FOR THAT DISEASE ENTITY EXIST OTHER THAN HSCT. –EXAMPLE-

Major Division of Disease Types by Country APBMT



JSHCT (Japan Society for Hematopoietic Cell Transplantation) has a guideline for the indications of HSCT. The guideline was made according to the evidences and consensus from oversea countries as well as our own data. In that guideline, there are categorization of HSCT indication.

D: definite

R: in routine use for selected patients

CRP: to be undertaken in approved clinical research protocol

NR: not generally recommended

Here, "D" and "R" can be considered to belong this category.

2. FEW THERAPEUTIC MODALITIES FOR THAT DISEASE STATUS EXIST OTHER THAN HSCT. –FROM JSHCT GUIDELINE-

Example

Indication of HSCT Aplasia JSHCT 2002

As 1st line	Age	Sibling	UR BM DNA	
			Match	Mismatch
VSAA	<40	D	NR	NR
SAA	<40	D/R	NR	NR
SAA	>40	R	NR	NR
NR to IST				
VSAA	Any	D	R/CRP	CRP
SAA	Any	D		

D: definite

R: in routine use for selected patients

CRP: to be undertaken in approved clinical research prot.

NR: not generally recommended

In the guideline of JSHCT, “CRP” can be considered to belong this category.

3. REASONABLY IMPROVED OUTCOME IN THAT DISEASE ENTITY/STATUS CAN BE EXPECTED BY HSCT. –FROM JSHCT GUIDELINE-

Example

Indication of HSCT Aplasia JSHCT 2002

As 1st line	Age	Sibling	UR BM DNA	
			Match	Mismatch
VSAA	<40	D	NR	NR
SAA	<40	D/R	NR	NR
SAA	>40	R	NR	NR
NR to IST				
VSAA	Any	D	R/CRP	CRP
SAA	Any	D		

D: definite

R: in routine use for selected patients

CRP: to be undertaken in approved clinical research prot.

NR: not generally recommended

Summary

1. Few therapeutic modalities for that disease entity exist other than HSCT.

- Hemoglobinopathy
- Other hereditary diseases

Wiskot-Aldrich, Dysgamma+cyclic neutropenia,
Congenital neutropenia, Kostmann, Chronic mucocut.neutropenia,
Combmbined Immunodeficiency, Hyper IgE syndrome,
CGD,Osteopetrosis, FEL,Hemophagocytic syndrome、I-cell disease,
Gaucher, Hurler-Scheie, Hunter, Sanfilippo,Morquio,
Marteux –Lamy, MPSⅦ, Mucopolysaccharidosis,
Adrenoleukodystrophy, Metach.leukodystrophy, GMI gangliosidosis, Pompe

Summary

2. Few therapeutic modalities for that disease status exist other than HSCT.

- Aplasia: (V)SAA (Except from UR as the first line)
- MDS: All types except IPSS low & Int-1 from UR at adult
- AML: All status except low risk at adult, low and standard risk at child in 1st CR
- ALL: All status except standard risk at adult, low and standard risk at child in 1st CR
- CML: Except to MPCR/CCR from UR

Summary
3.Reasonably improved outcome in that disease entity/status can be expected by HSCT.

- Aplasia: NR to IST, (V)SAA from DNA mismatch at adult, moderate from sibling at child
- MDS: Low, Int-1 from matched UR
- AML: t(15;17) from sibling, Low risk from sibling or matched UR
- ALL: Standard risk in 1st CR from sibling or matched UR at adult
- CML: BC from DNA mismatch UR