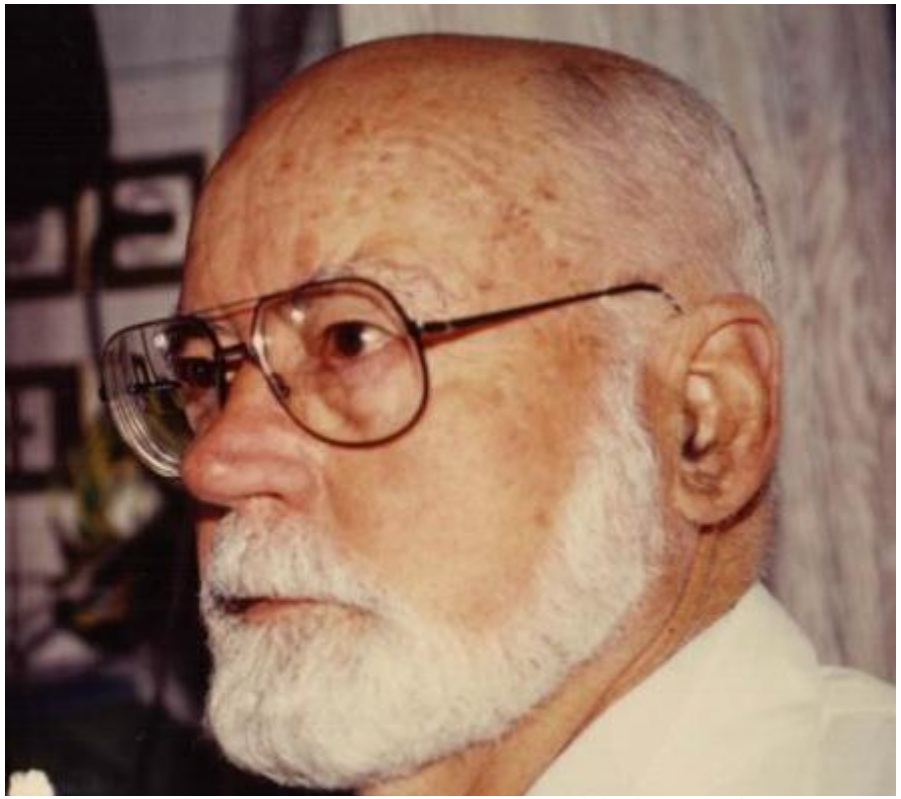
Global Perspectives of HSCT:
Past, Present and Future

Dietger Niederwieser, MD

LABMT meeting: Salvador, Brazil

October 2013

History of HSCT



INTRAVENOUS INFUSION OF BONE MARROW IN PATIENTS RECEIVING RADIATION AND CHEMOTHERAPY*

E. DONNALL THOMAS, M.D.,† HARRY L. LOCHTE, JR., M.D.,‡ WAN CHING LU, PH.D.,§ AND JOSEPH W. FERREBEE, M.D.¶

COOPERSTOWN, NEW YORK, AND BOSTON, MASSACHUSETTS

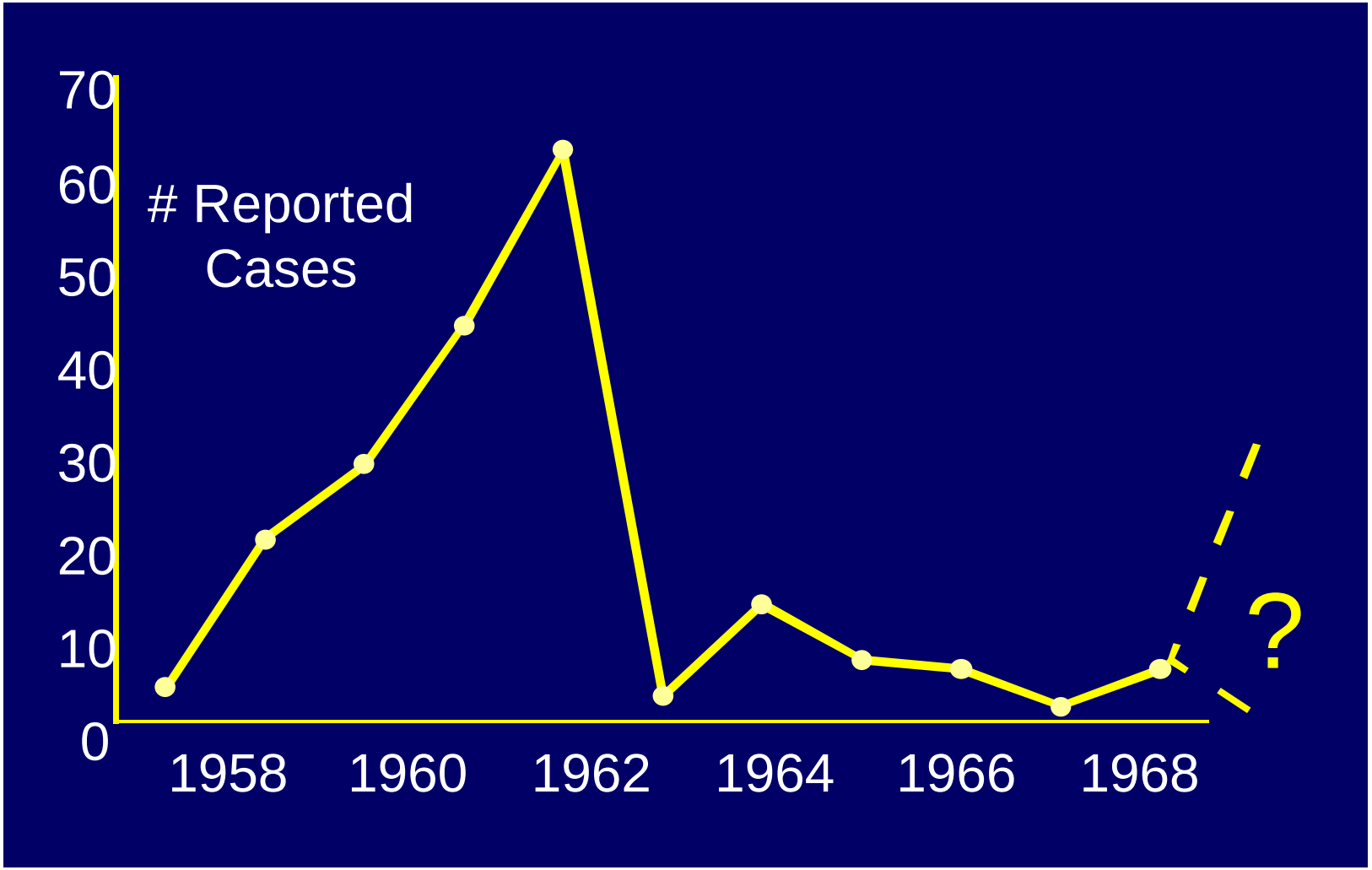
NEJM 257, 491-496, 1957

Bone Marrow Transplantations 1958 - 68

Diseases	# Patients			
	Total	Graft Failure	GVHD	Alive
Aplastic anemia	73	66	5	0
Hematologic malignancies	115	56	33	0
Immunodeficiencies	15	3	9	3
Total	203	125	47	3

Bortin, *Transpl* 9: 571, 1970

Bone Marrow Transplantations 1958 - 68



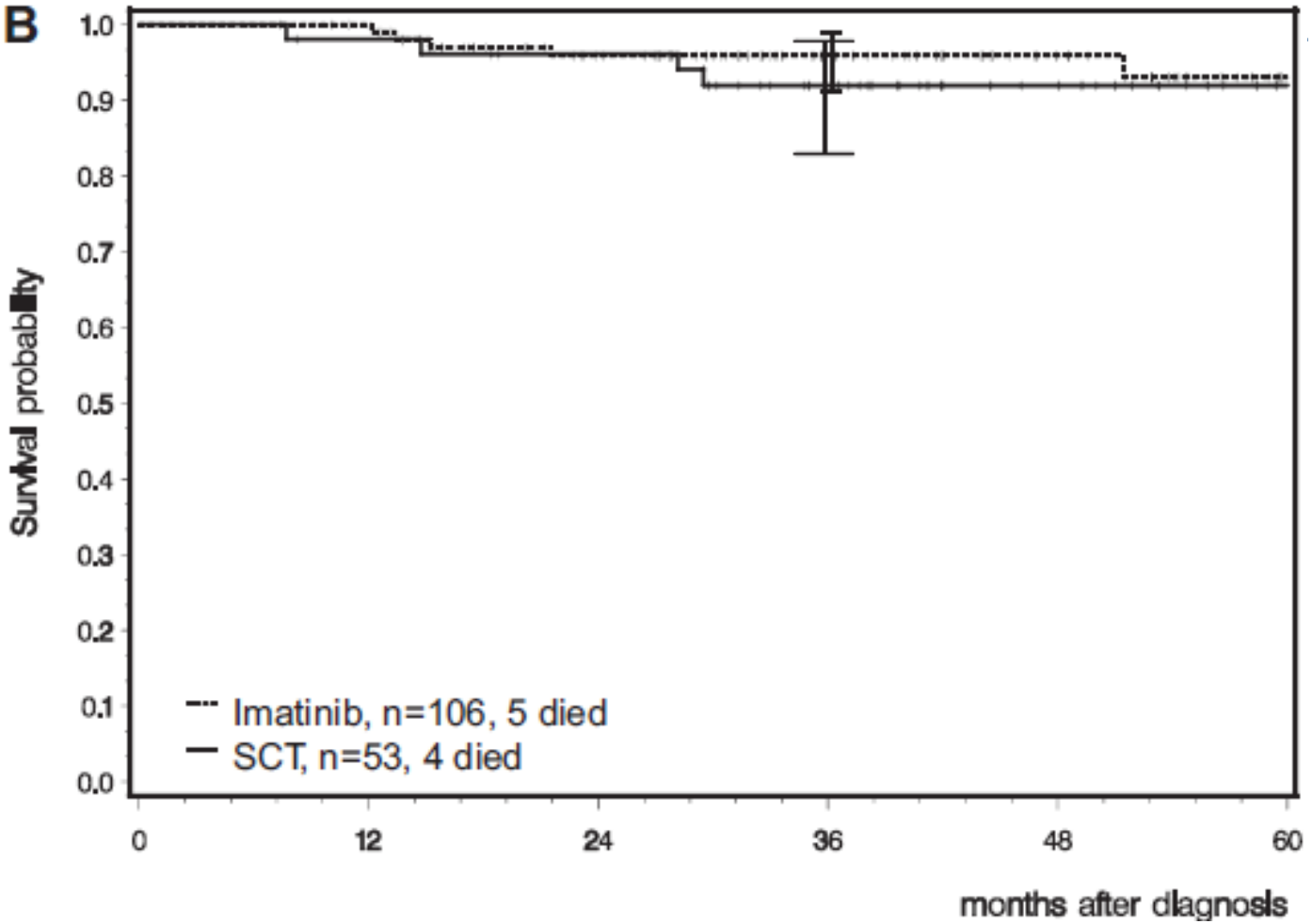
Bortin, *Transpl* 9: 571, 1970

Milestones in the Development of HSCT from 1957 to 2012

	1957-1970	1971-1985	1986-1991	1992-1995	1996-2005	2006 - 2012 est.	Total
Milestones	Early phase	10 000	50 000	100 000	500 000	1 000 000	
running total	275	9323	49099	128054	535603	953651	
Total HSCT	275	9048	39776	78955	407549	418048	953651
Allogeneic	275	7022	20559	30379	154478	187588	400301
Autologous	0	2026	19217	48576	253071	230460	553350
AMR/PAH Total	271	2422	14975	33734	126212	119140	296754
Allogeneic	271	2375	7242	12092	51347	54437	127764
Autologous	0	47	7733	21642	74865	64703	168990
SEAR/WPR Total	0	505	3349	9120	53763	73342	140079
Allogeneic	0	450	2508	5061	30340	44607	82966
Autologous	0	55	841	4059	23423	28735	57113
EMR/AFR Total	0	33	300	441	5104	9625	15503
Allogeneic	0	32	239	357	3821	5968	10417
Autologous	0	1	61	84	1283	3657	5086
EUR Total	4	6088	21152	35660	222470	215941	501315
Allogeneic	4	4165	10570	12869	68970	82576	179154
Autologous	0	1923	10582	22791	153500	133365	322161

Networking – WBMT Federation

Results of HSCT in chronic myelogenous leukemia



Saussele et al, Blood 2010

Results of HSCT in high risk acute leukemia

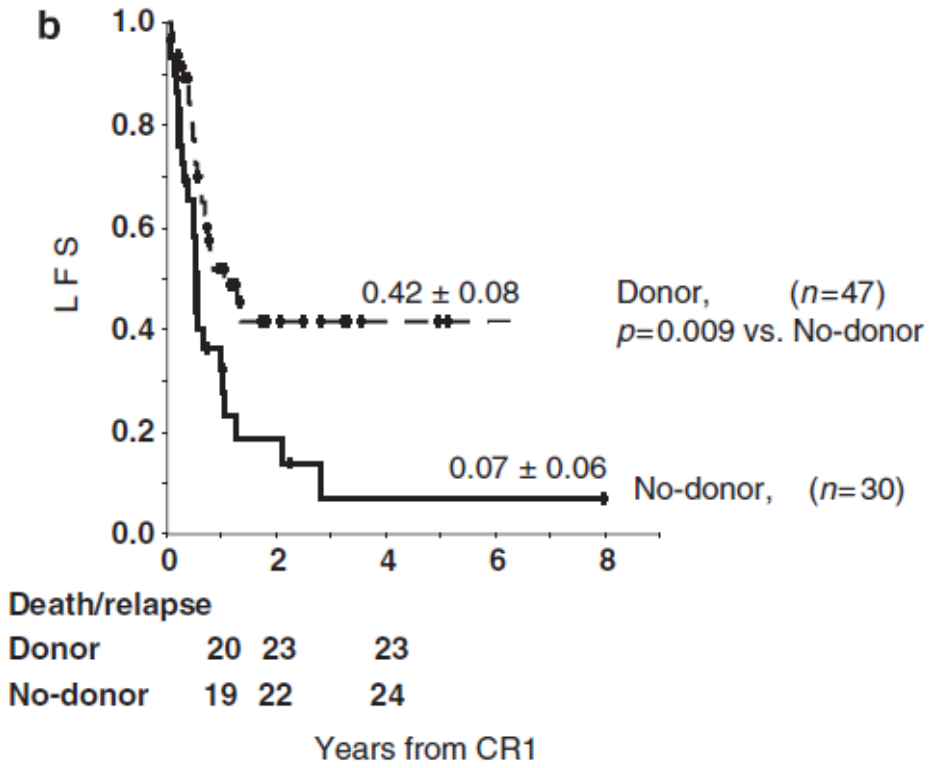


Figure 2 Overall survival (**a**) and leukaemia-free survival (**b**) of patients with acute myeloid leukaemia (AML) in complete remission (CR)1 according to donor and no donor analysed as intention to treat at CR1 ($n=77$). Numbers of events at different time points from CR1 are given for the donor and the no-donor group.

Basara et al, Leukemia, 2009

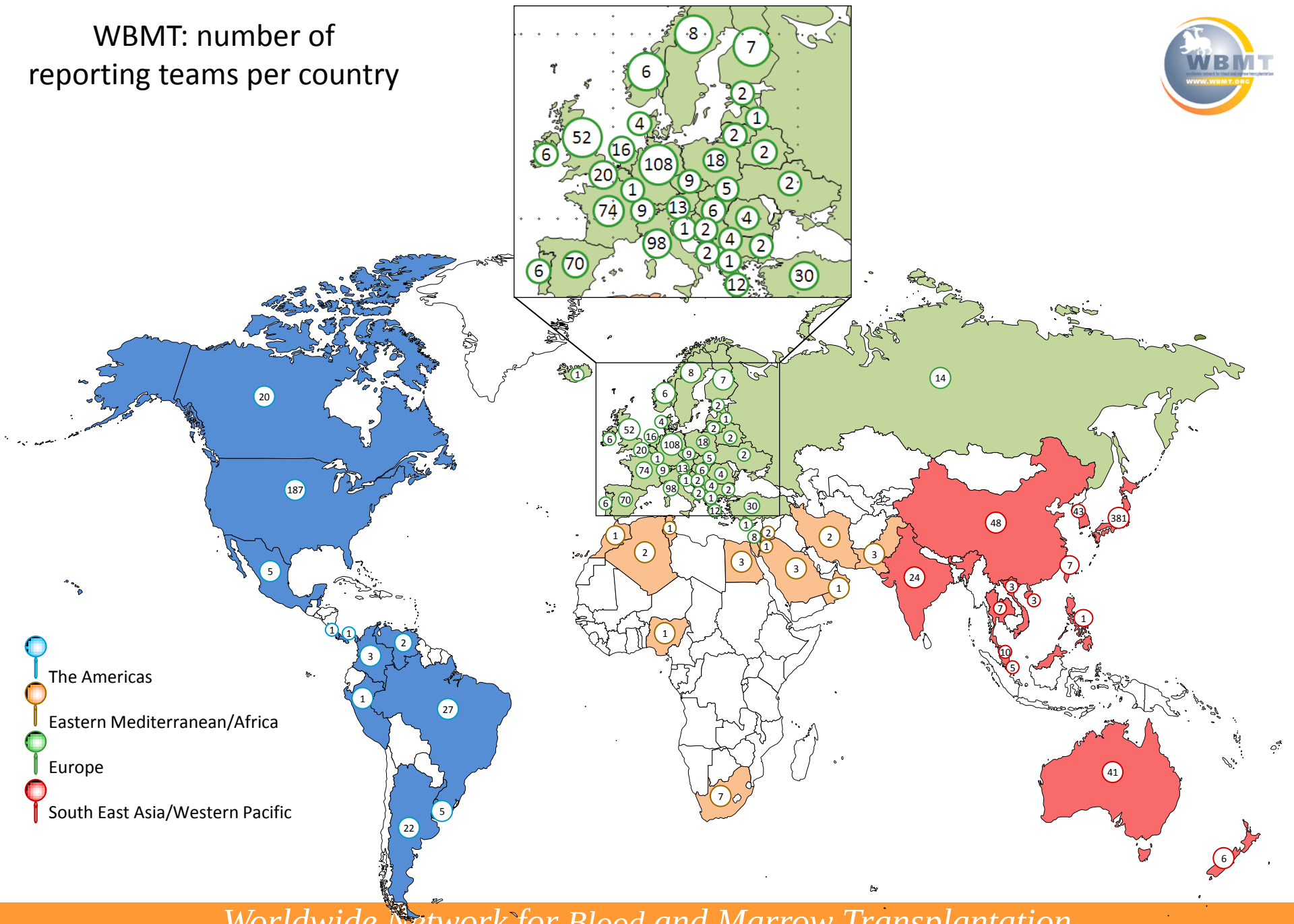
Why do we need WBMT?

- Promote excellence in HSCT incl. cellular therapies (e.g. by registering activities, fostering global studies)
- Global Platform for national authorities / regulators
- Exchange knowledge / experience around the world
- Expensive procedure

Standing Committees

- AHCTA / Accreditation
- Donor Issues
- Transplant Center and Recipient Issues
- Education and Dissemination
- Graft Processing

WBMT: number of reporting teams per country



Deliverables

- EM(E)A meeting Antwerpen (10/10)

  Interaction with Agency
- Bologna meeting (2/11)

  Exploring Vigilance notification for organs, tissues and cells
- Bruxelles meeting DG Sanco (2/11)

  Consultation on labeling
- Vietnam meeting (11/11):

  Encourage integration of HSCT within the Healthcare Policies of developing countries
- One million transplant

  Press release around the world

Deliverables

- Survey 2006

JAMA®

Online article and related content current as of May 7, 2010.

Hematopoietic Stem Cell Transplantation: A Global Perspective

Alois Gratwohl; Helen Baldomero; Mahmoud Aljurf; et al.

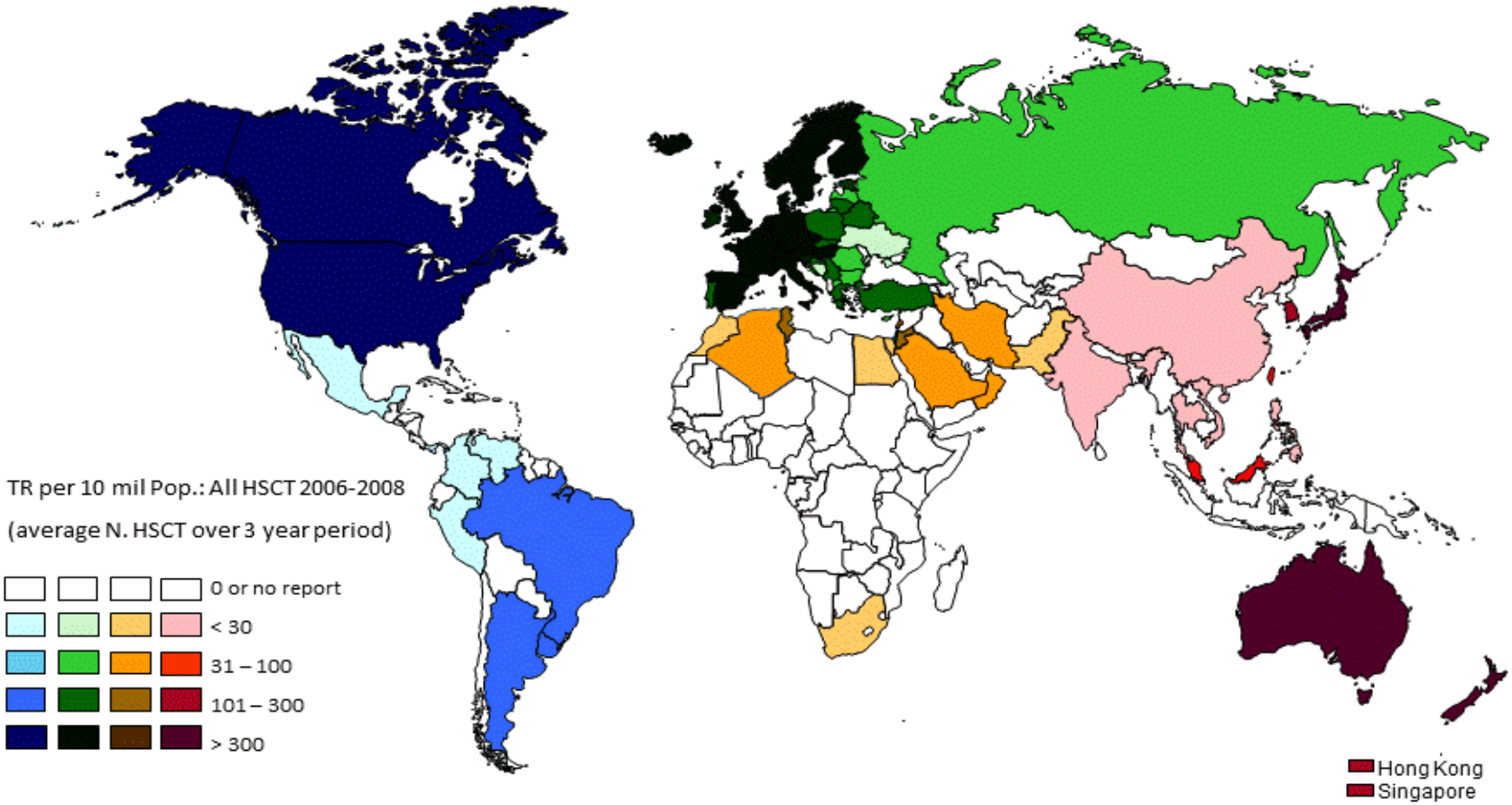
JAMA. 2010;303(16):1617-1624 (doi:10.1001/jama.2010.491)

- Survey 2007 – 2008

haematologica
the hematology journal

- One million transplant manuscript (in preparation)
- Global Transplant Center Number (GTCN)

Global HSCT activity



Global Survey 2010 overview

WHO Regions	4
Countries	75 (68 with data)
Teams	1440
Year of Transplant	2010 (preliminary data)
Patients	57 622 26 758 allogeneic (46%) 30 864 autologous (54%)
Donor type	12 567 family (47%) 14 191 unrelated (53%) (2912 cord blood)

09/10 preliminary data

Transplant Type by Region: 2010

Main indication	Allogeneic HSCT	Autologous HSCT	Total
Europe	11 518	17 137	28 655 (50%)
The Americas	7 475	8 920	16 395 (28%)
South East Asia / Western Pacific	6 911	4 244	11 155 (20%)
Eastern Mediterranean / Africa	854	563	1 417 (2%)
Total	26 758 (46%)	30 864	57 622

68 reporting countries
1440 contributing teams

09/10 preliminary data

The first SCT

Cortesy Dr. Bujan

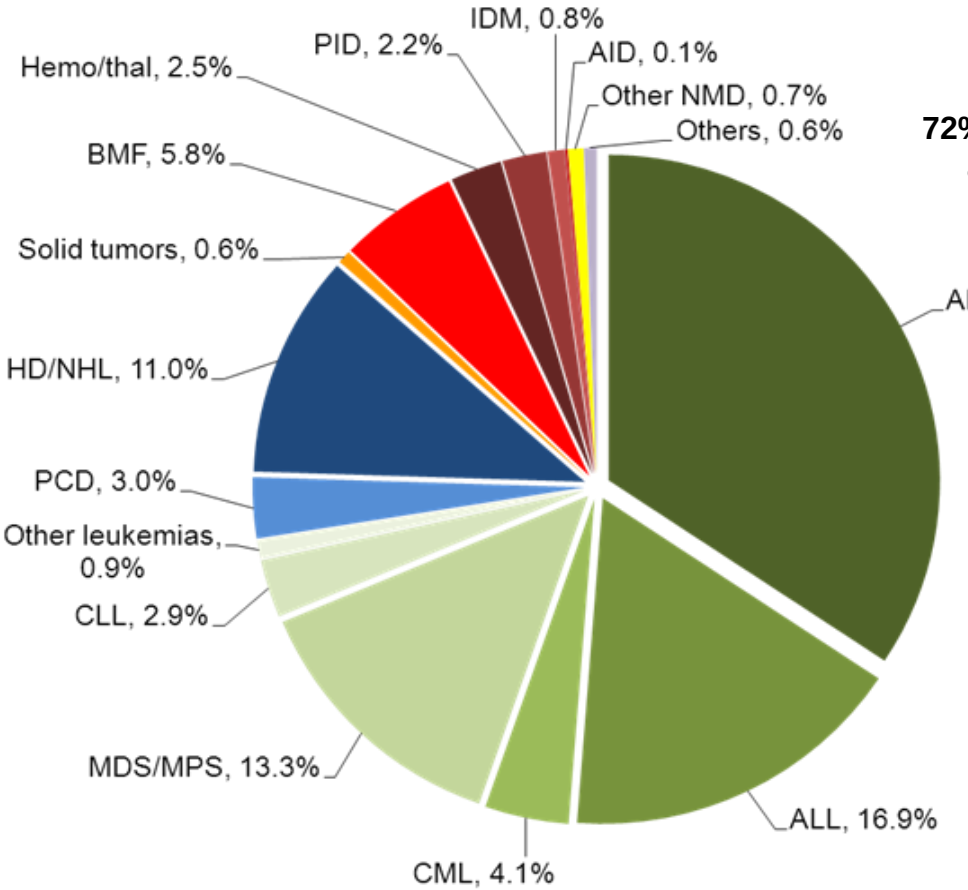
WBMT/LABMT

Numbers of centers in each LABMT Country

Country	N teams LABMT	WBMT N teams reporting in 2009/10
Argentina	24	22
Brazil	69	17
Chile	7	
Columbia	13	1
Costa Rica	3	1
Cuba	5	
Ecuador	3	
Mexico	21	5
Panama	3	
Peru	5	1
Dominican Republic	1	
Uruguay	4	1
Venezuela	2	
Total	160	48

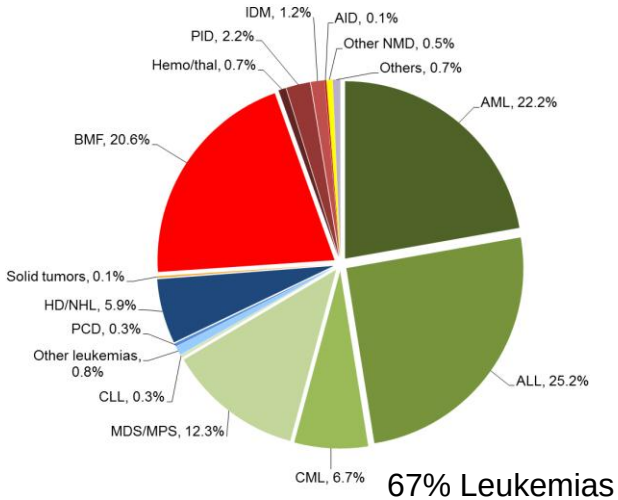
Main Indications: Allogeneic HSCT

26 758 HSCT in 2010



72% of allogeneic HSCT are for Leukemias

LABMT 729 HSCT in 2010

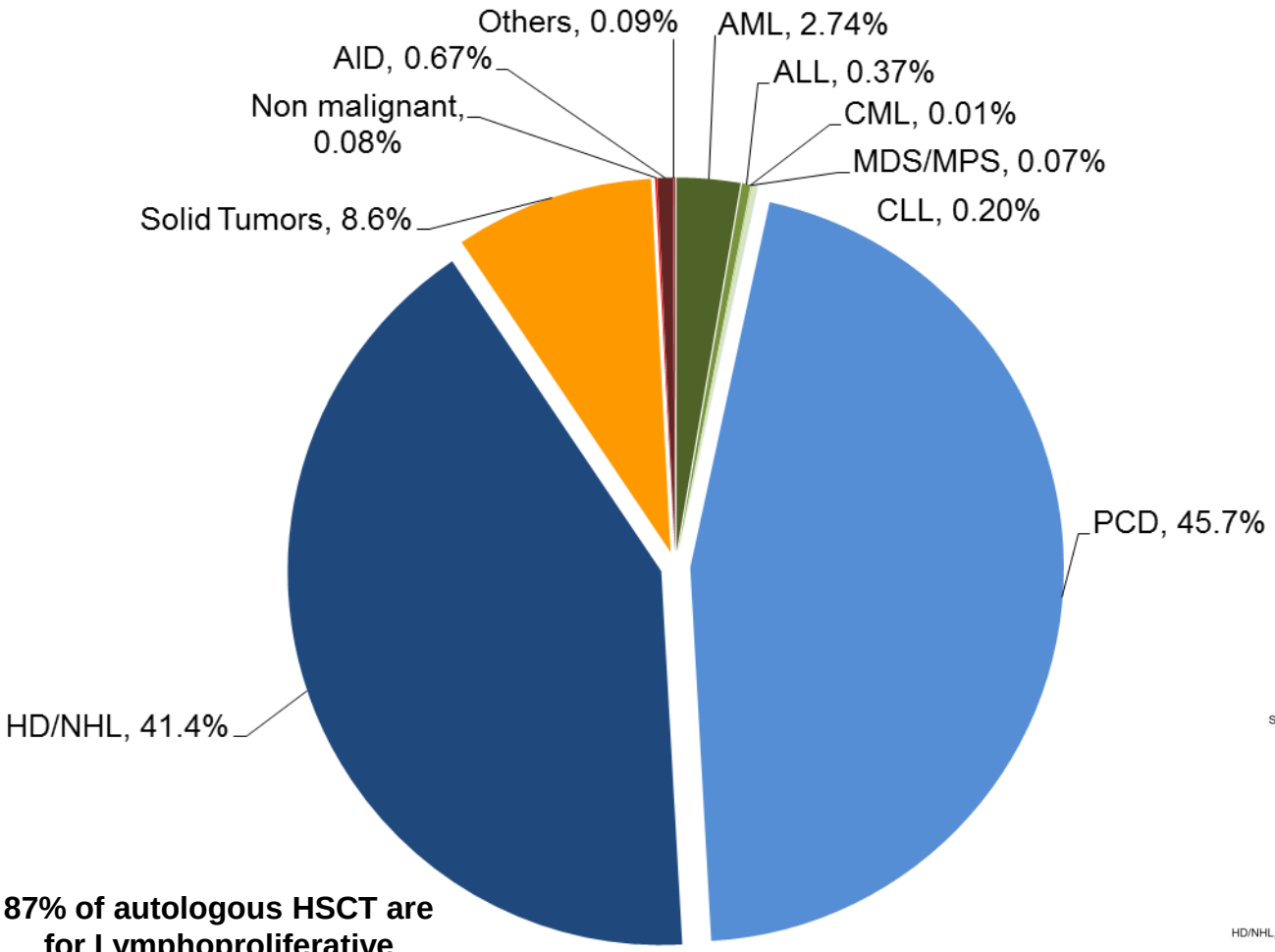


67% Leukemias

09/10 preliminary data

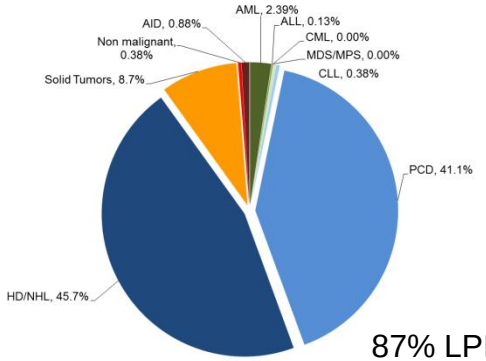
Main Indications: Autologous HSCT

30 864 HSCT in 2010



87% of autologous HSCT are for Lymphoproliferative disorders

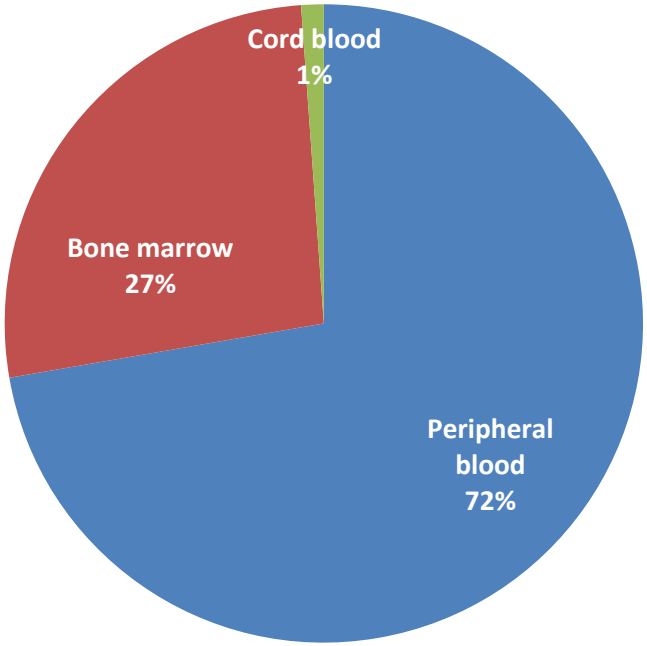
LABMT 795 HSCT in 2010



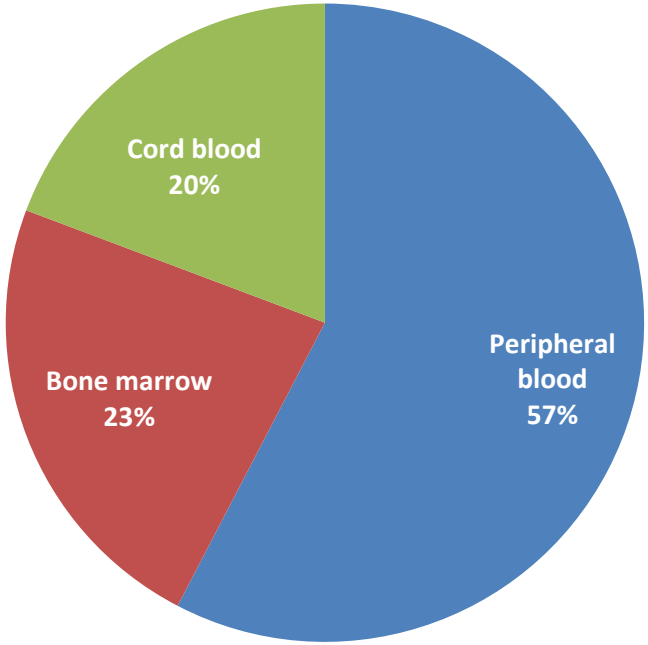
87% LPD

09/10 preliminary data

Stem cell source in 2010



Family

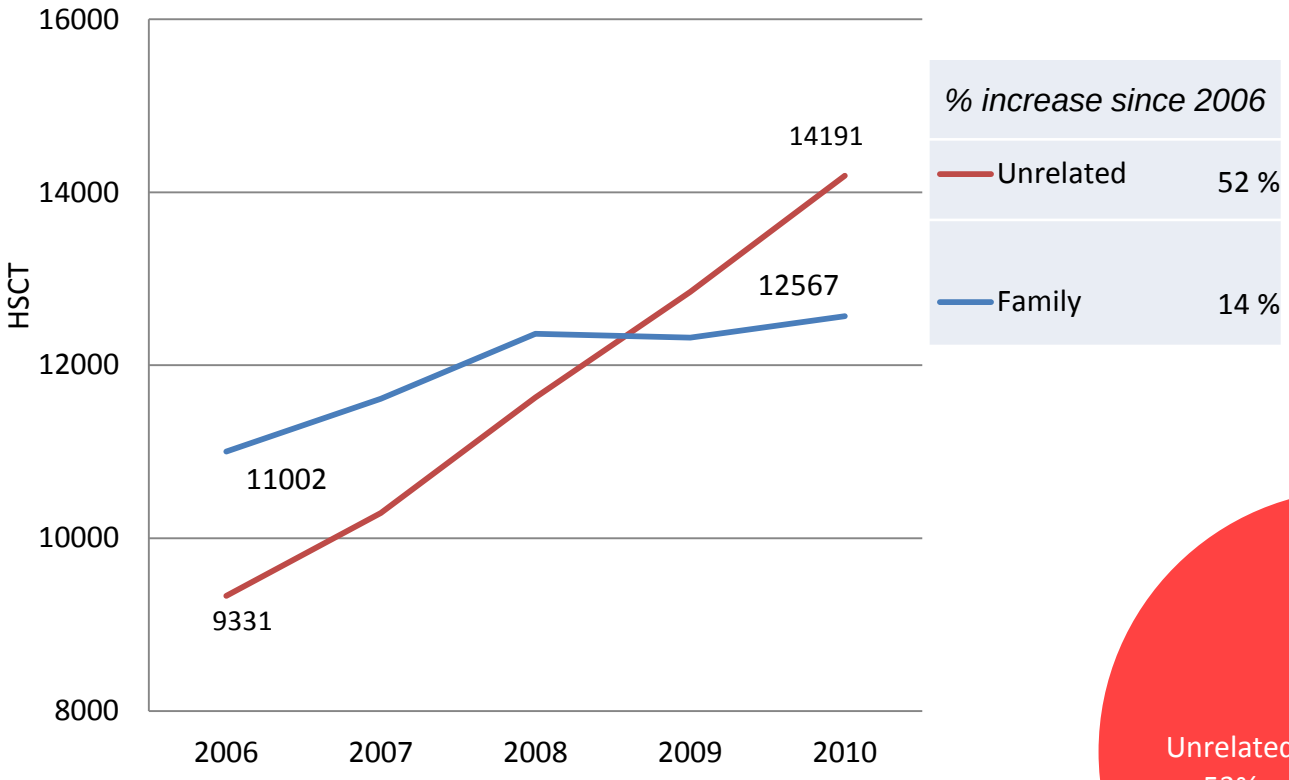


Unrelated

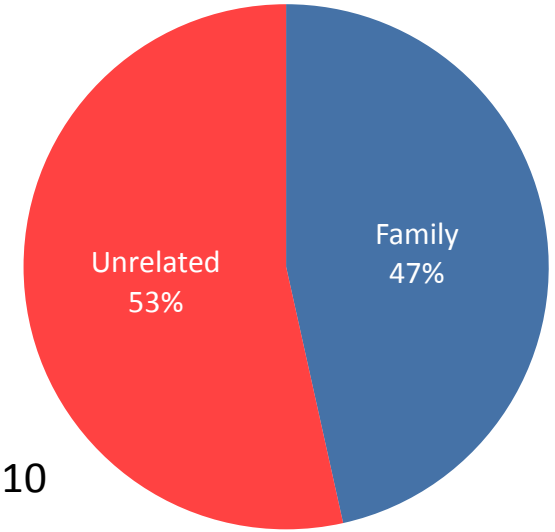
2010	Family	Unrelated
Peripheral blood	8973	8110
Bone marrow	3441	3322
Cord blood	153	2759

09/10 preliminary data

Trend over 5 years : Donor type



% increase since 2006	
Unrelated	52 %
Family	14 %



2010

09/10 preliminary data

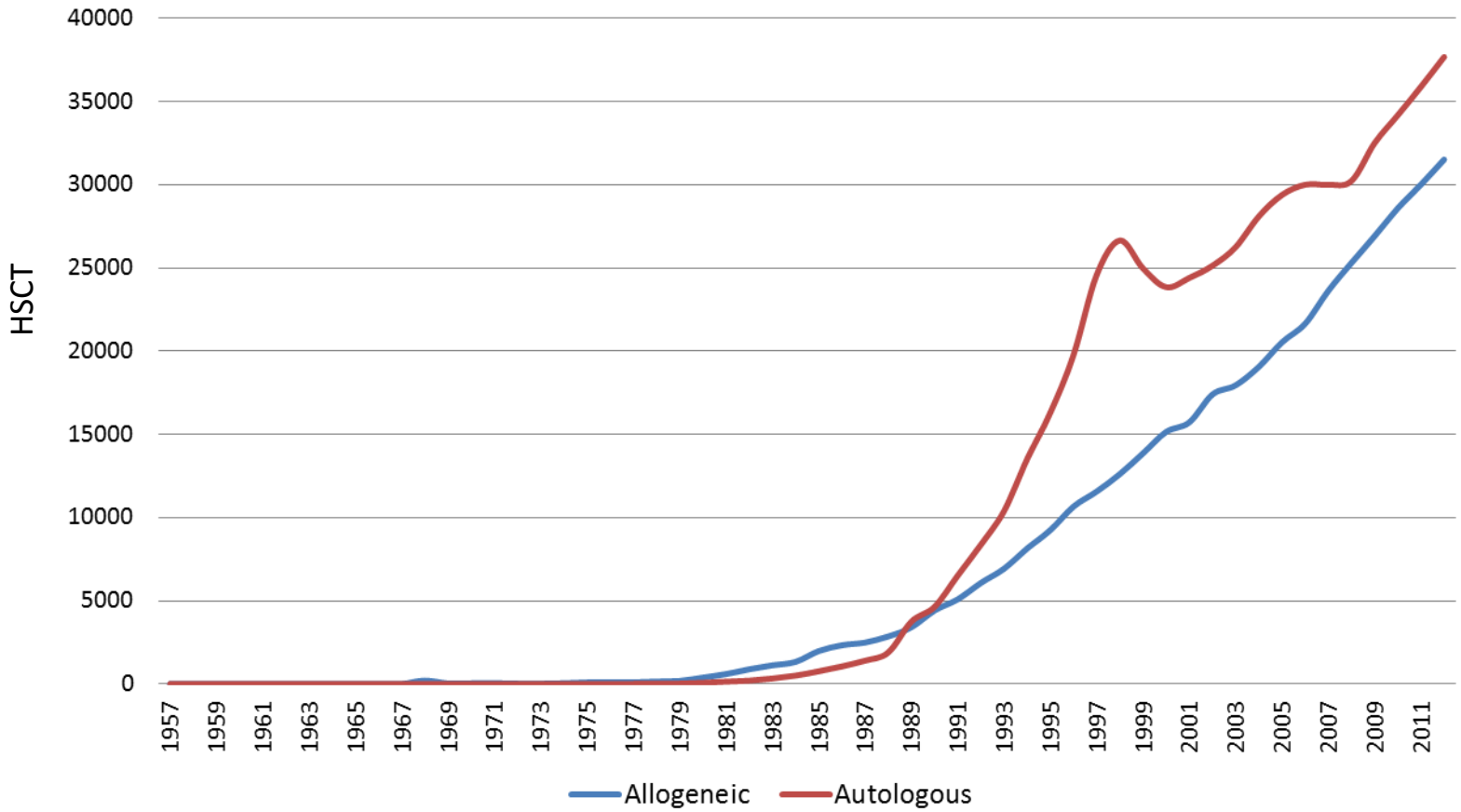
Global Activity Survey: 2006 – 2010

Allogeneic	**2006**	**2009**	**2010**		
Acute Leuk/MDS/MPS	12 502	16 070	17 531	↑	40%
Chronic Leuk	1 890	1 693	1 865	-	
Lymphoproliferative disorders	3 219	3 742	3 762	↑	17%
Solid Tumors	150	152	169	-	
Non Malignant disorders	2 360	3 973	3 266	↑	38%
BMF	*1 292*	*1 413*	*1 564*	↑	21%
Others	212	102	165	↓	
Total	20 333	24 732	26 758	↑	32%
Autologous					
Leukemias	1 726	1 169	1 052	↓	39%
PCD	10 675	12 732	14 103	↑	32%
Lymphomas	10 980	12 349	12 792	↑	17%
Solid Tumors	2 560	2 495	2 658	-	
Non Malignant disorders	193	229	231	-	20%
Others	96	28	28	↓	
Total	26 230	29 001	30 864	↑	16%
Total	**46 563**	**53 734**	**57 622**	↑	24%

2010 preliminary data

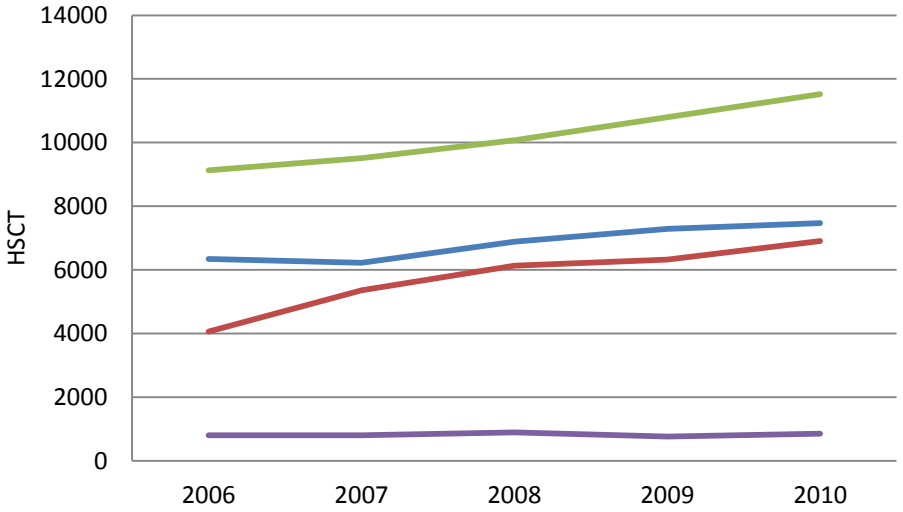
Global Transplant Numbers:

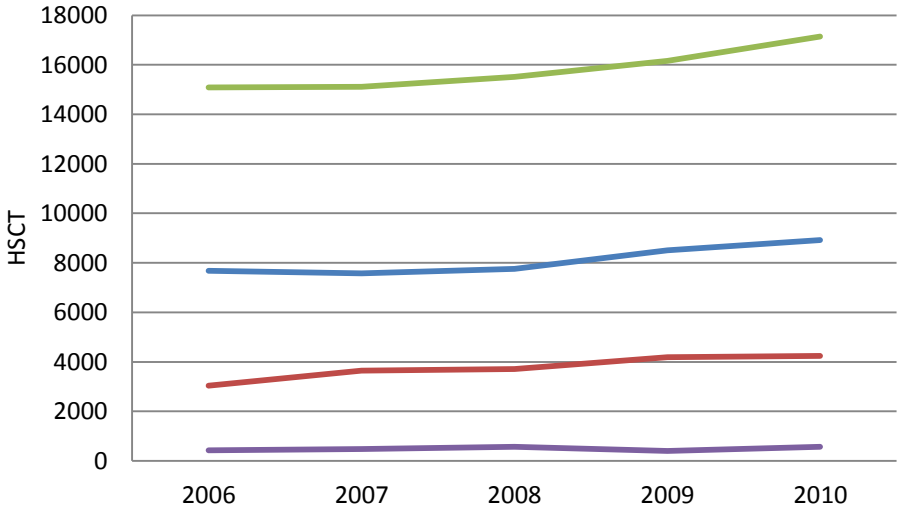
Allogeneic and autologous



preliminary data

Trend over 5 years by region





Allogeneic HSCT: % increase since 2006	
EUR	26 %
AMR/PAH	18 %
SEAR/WPR	70 %
EMR/AFR	6 %

Autologous HSCT: % increase since 2006	
EUR	14%
AMR/PAH	16 %
SEAR/WPR	40%
EMR/AFR	32 %

09/10 preliminary data

Conclusions

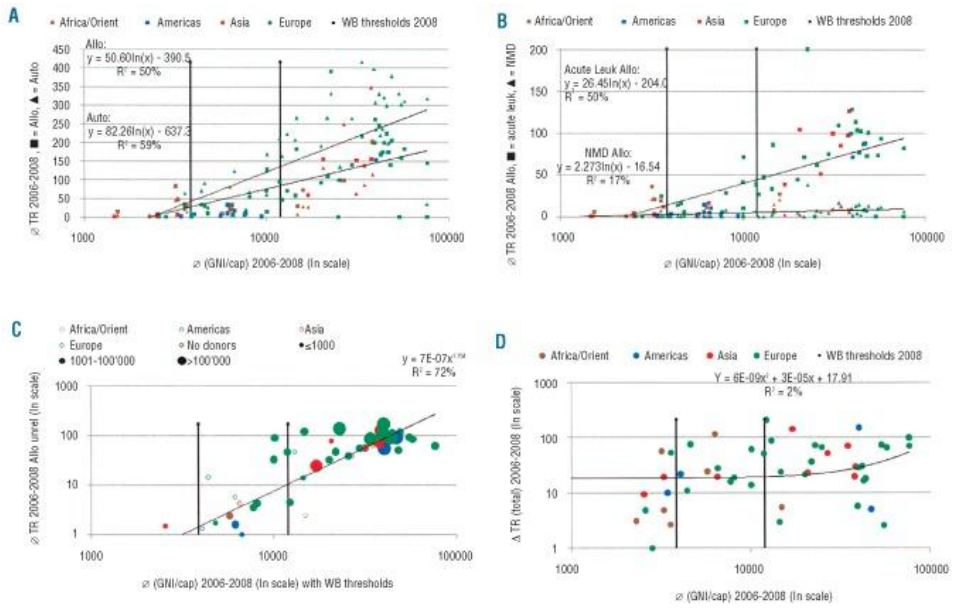
- Increasing HSCT activities around the world
- Difference in activities between regions
- Networking and cooperation with WHO and national authorities of fundamental importance
- Experience is the major hurdle for success
- Salvador Meeting will be a key tool for WBMT mission
- Reporting essential part of a HSCT

Thank you

Worldwide Network for Blood and Marrow Transplantation

Some of the Key Research questions

- Country / regional level
  - Macro economics
  - GNI, health care systems
  - Deveoplemental status



Niederwiesser et al. Hematologica 2013;98(8): 1282-1290

- Are there differences in HSCT use?
  - If yes
    - Quantity?
    - Choices available?
  - If yes
    - Factors associated with differences?
      - Economical
      - Availabilty of centres
      - Expectation (CML, breast cancer)
      - Evidence based medicine
      - .....