

# Importance of Outcomes Databases

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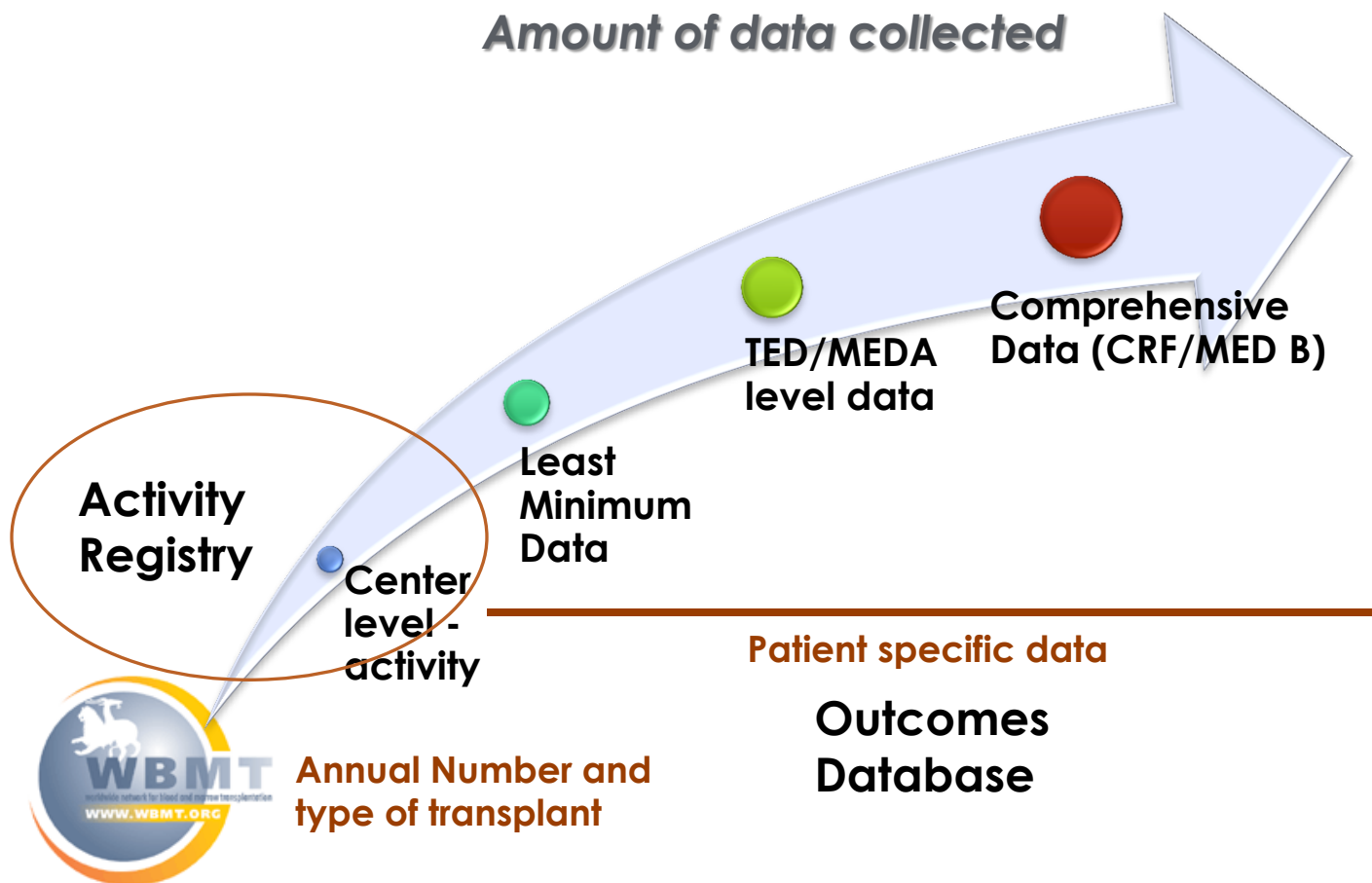
*Worldwide Network for Blood and Marrow Transplantation*

# The Value of Global Outcome Registries : Pros and Cons

- **Helps health policy-makers**
  - Epidemiological data on SCT and evolution with time
  - Evaluation of clinical practice: centre effect
  - Impact of socio-economic and geographical factors
- **Provides hypothesis-generating information to health outcomes researchers**

# Levels of data collection

*Amount of data collected*

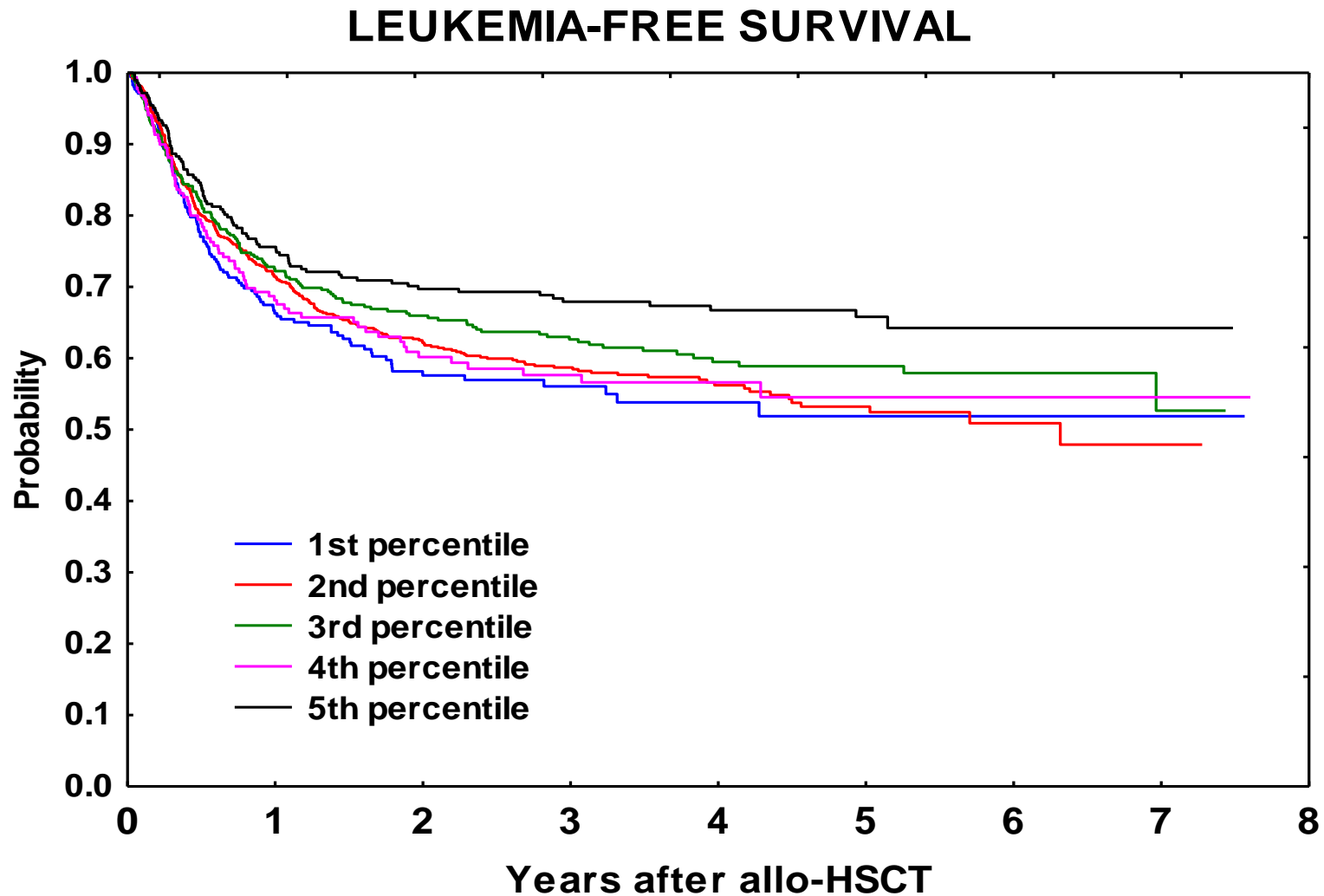


Annual Number and type of transplant

Patient specific data

Outcomes Database

# The Value of Global Outcome Registries : Pros and cons

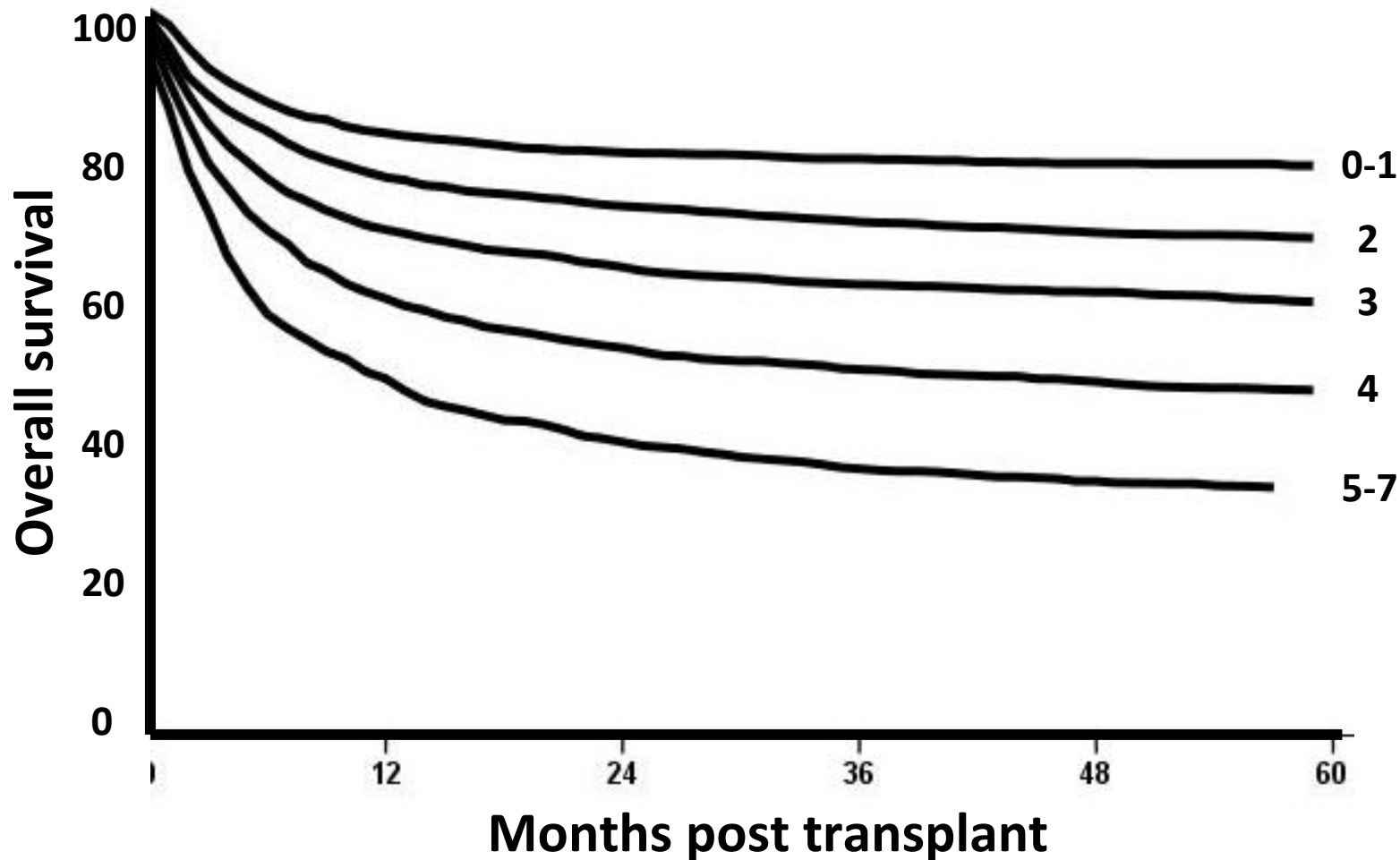


Association of Human Development Index with rates and outcomes of hematopoietic stem cell transplantation for patients with acute leukemia (Giebel et al, Blood 2010)

## Helps clinical decision making

- Defines trends in activity, e.g. increased use and success in older patients
- Identifies factors affecting outcome, eg. age, disease stage, conditioning regimens
- Determines outcomes for rare diseases and new indications
- Determines efficacy of various donor types and graft sources
- Assesses long-term quality of life and late complications

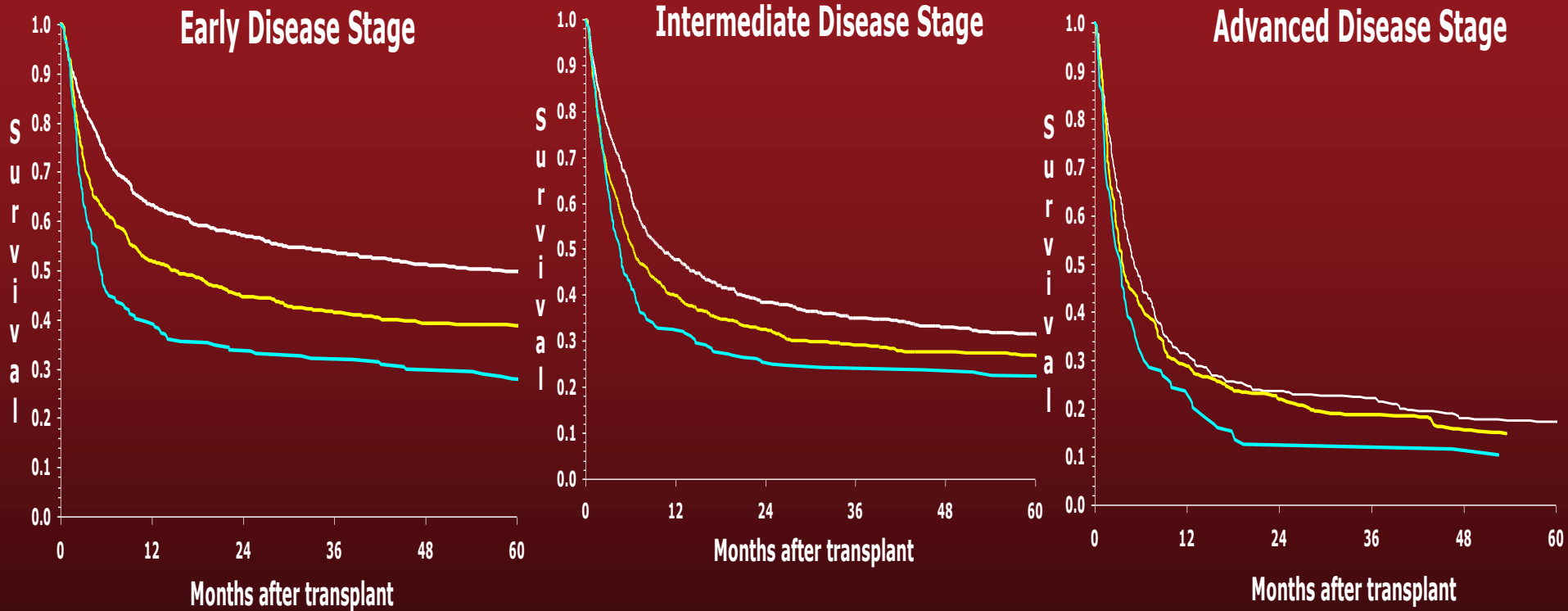
# The Value of Global Outcome Registries : Pros and cons



Outcome of allo-SCT for CML by EBMT risk score (2011), courtesy of Ronald Brand

# Influence of HLA matching

— 8/8 Match — 7/8 Match — 6/8 Match



Impact of single locus mismatch at A, B, C and DRB1:  
changed the paradigm for selecting adult donors

# The Value of Global Outcome Registries : Pros and cons

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ARTICLES

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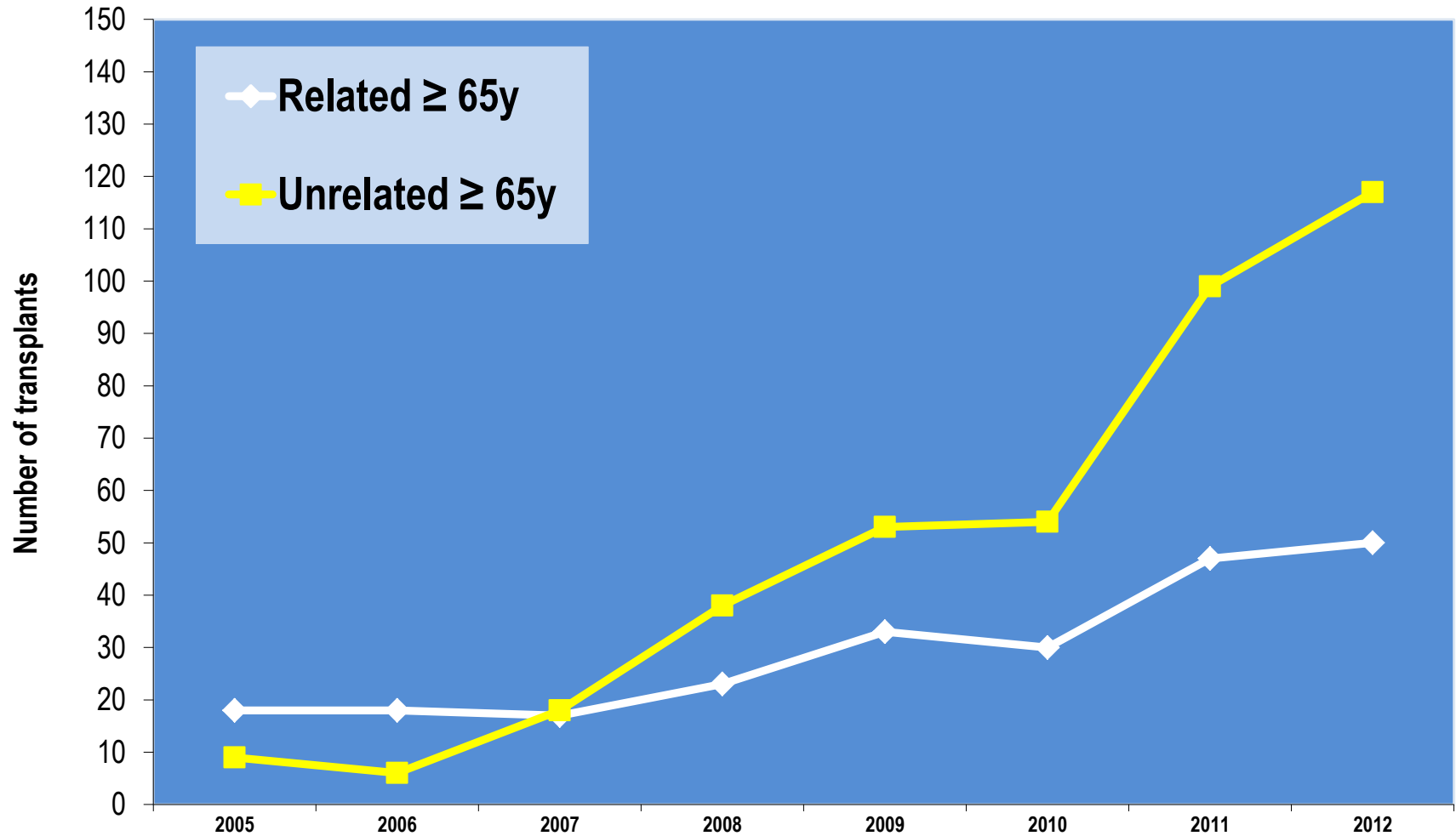
## **Pregnancy outcomes after peripheral blood or bone marrow transplantation: a retrospective survey**

*N Salooja, R M Szydlo, G Socie, B Rio, R Chatterjee, P Ljungman, M T Van Lint, R Powles, G Jackson, M Hinterberger-Fischer, H J Kolb, J F Apperley, for the Late Effects Working Party of the European Group for Blood and Marrow Transplantation*

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# The Value of Global Outcome Registries : Pros and cons



**Allografting for MDS in the USA: effect of implementing study protocol compatible with requirement of Medicare/CMS**

# The Value of Global Outcome Registries : Pros and cons

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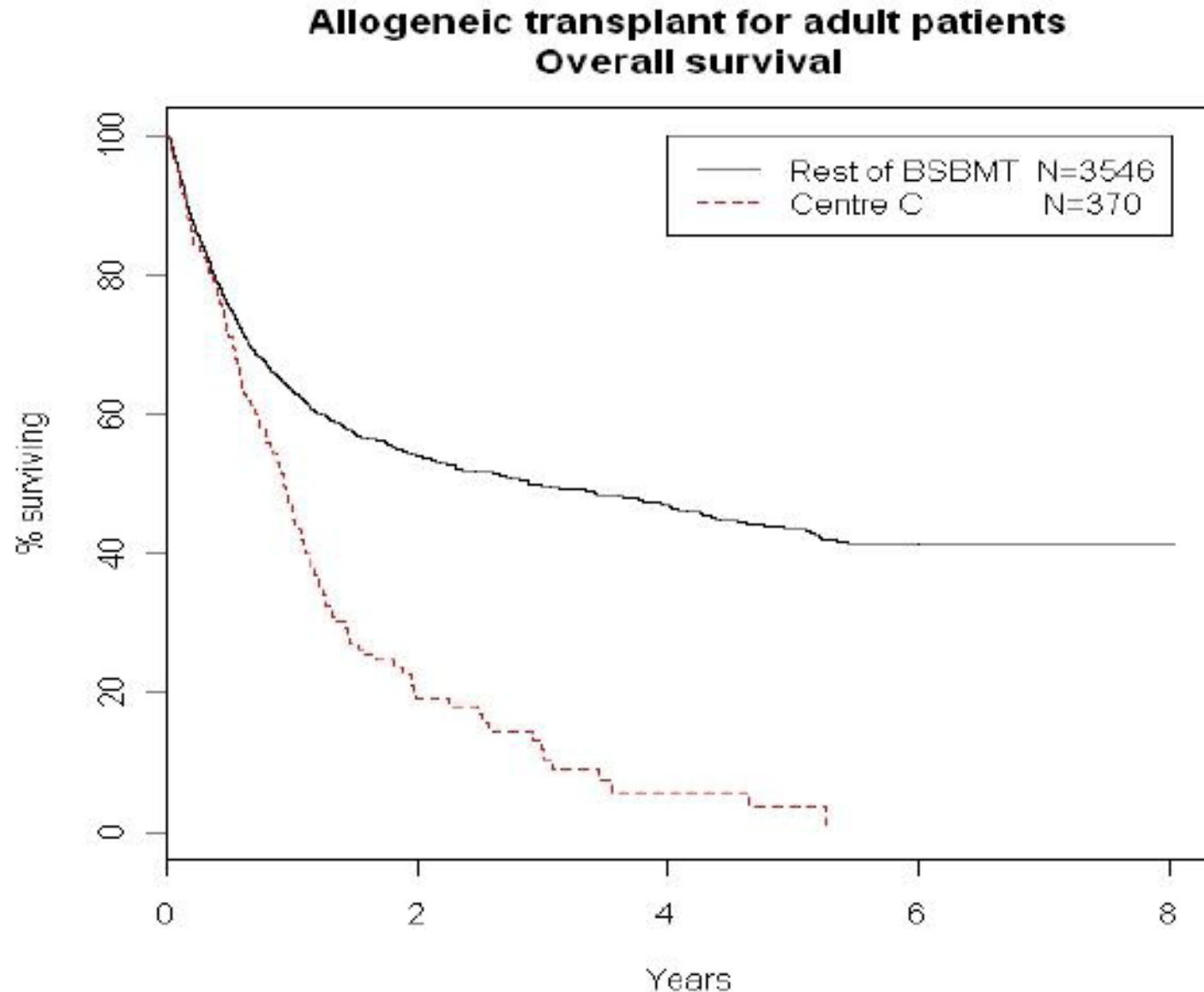
### Outcomes after Transplantation of Cord Blood or Bone Marrow from Unrelated Donors in Adults with Leukemia

Mary J. Laughlin, M.D., Mary Eapen, M.B., B.S., Pablo Rubinstein, M.D., John E. Wagner, M.D., Mei-Jei Zhang, Ph.D.,  
Richard E. Champlin, M.D., Cladd Stevens, M.D., Juliet N. Barker, M.D., Robert P. Gale, M.D., Ph.D.,  
Hillard M. Lazarus, M.D., David I. Marks, M.D., Ph.D., Jon J. van Rood, M.D.,  
Andromachi Scaradavou, M.D., and Mary M. Horowitz, M.D.

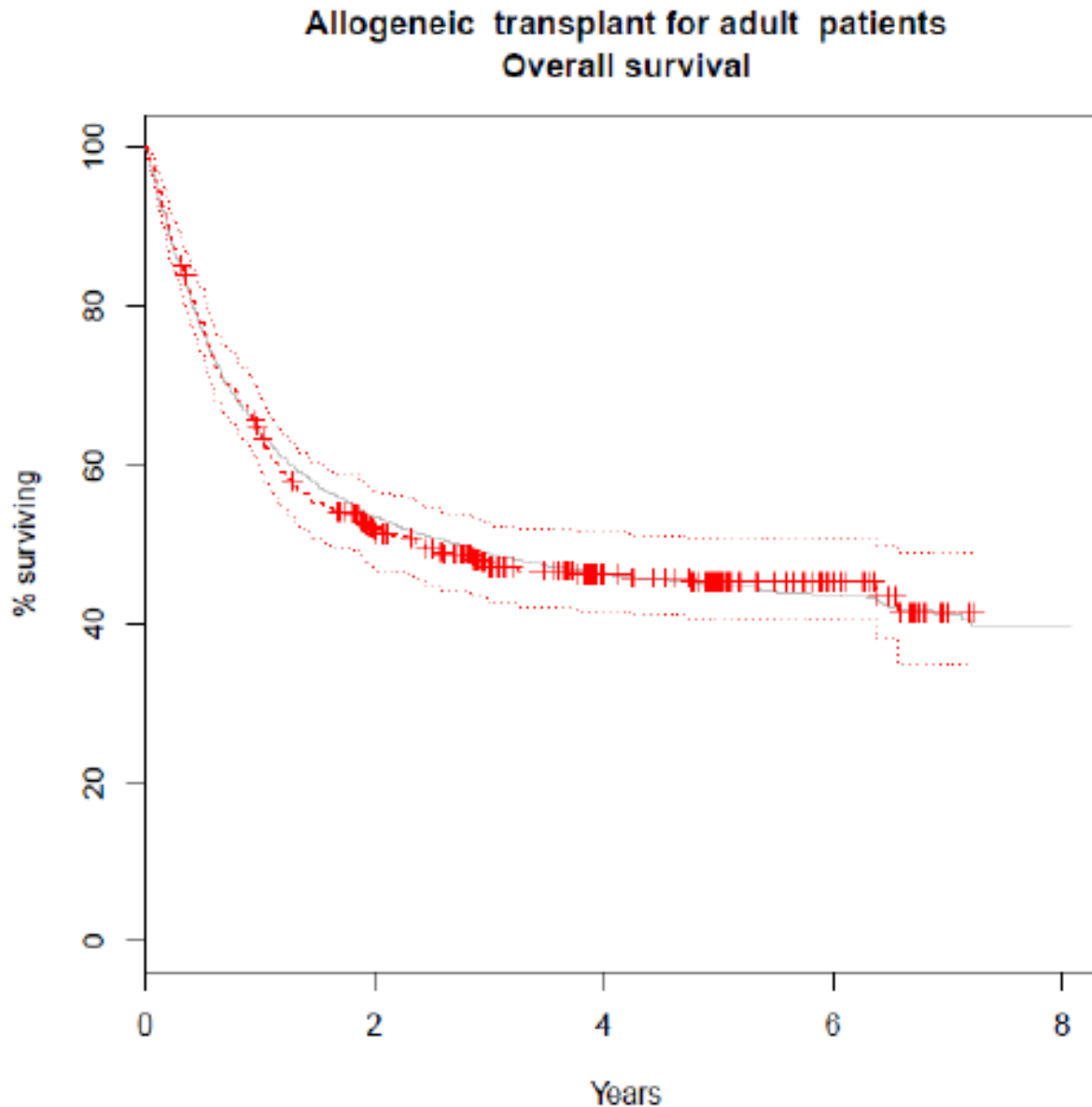
### Transplants of Umbilical-Cord Blood or Bone Marrow from Unrelated Donors in Adults with Acute Leukemia

Vanderson Rocha, M.D., Ph.D., Myriam Labopin, M.D., Guillermo Sanz, M.D.,  
William Arcese, M.D., Rainer Schwerdtfeger, M.D., Alberto Bosi, M.D.,  
Niels Jacobsen, M.D., Tapani Ruutu, M.D., Marcos de Lima, M.D., Jürgen Finke, M.D.,  
Francesco Frassoni, M.D., and Eliane Gluckman, M.D.,  
for the Acute Leukemia Working Party of European Blood  
and Marrow Transplant Group and the Eurocord–Netcord Registry\*

# The Value of Global Outcome Registries : Pros and Cons

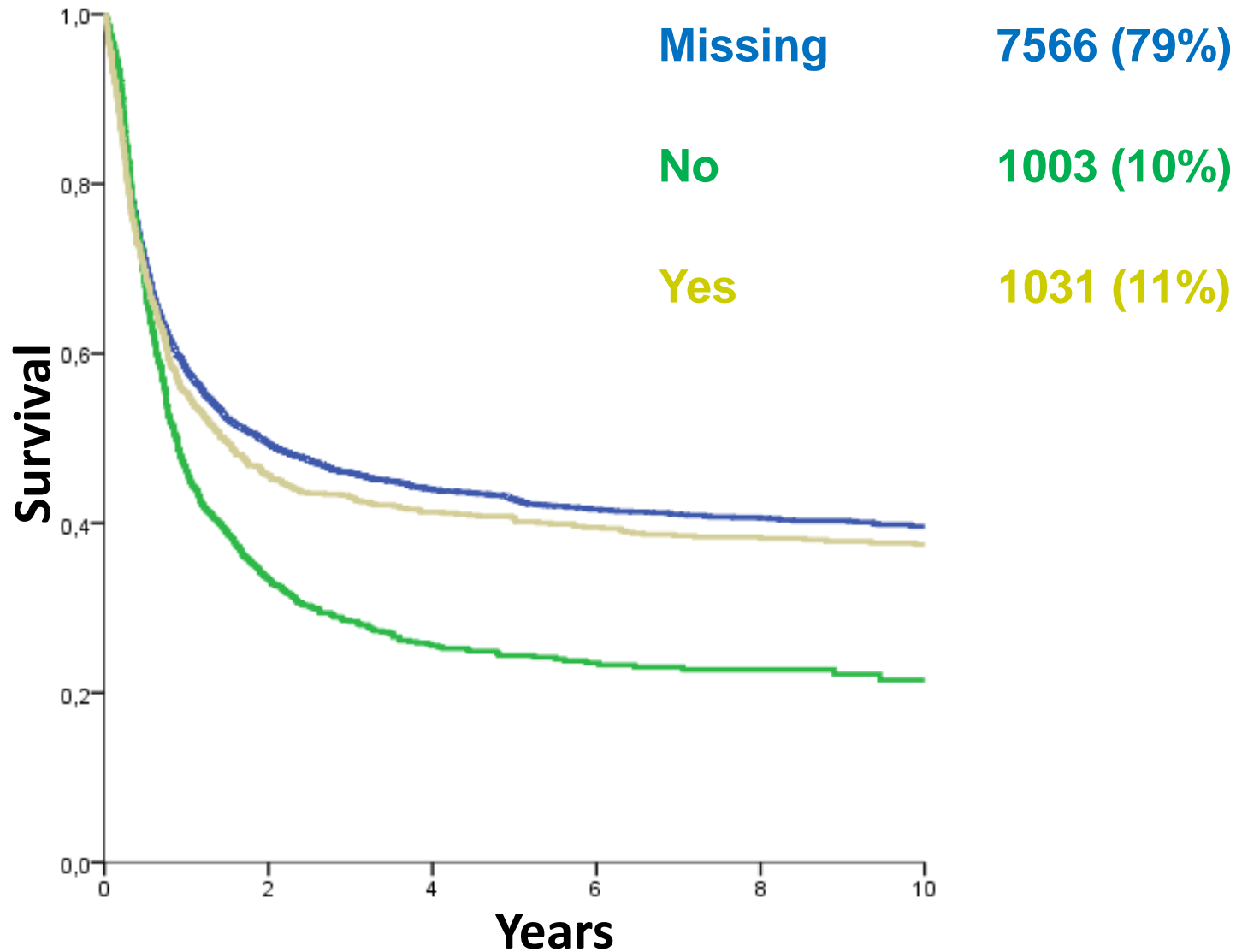


# The Value of Global Outcome Registries : Pros and Cons



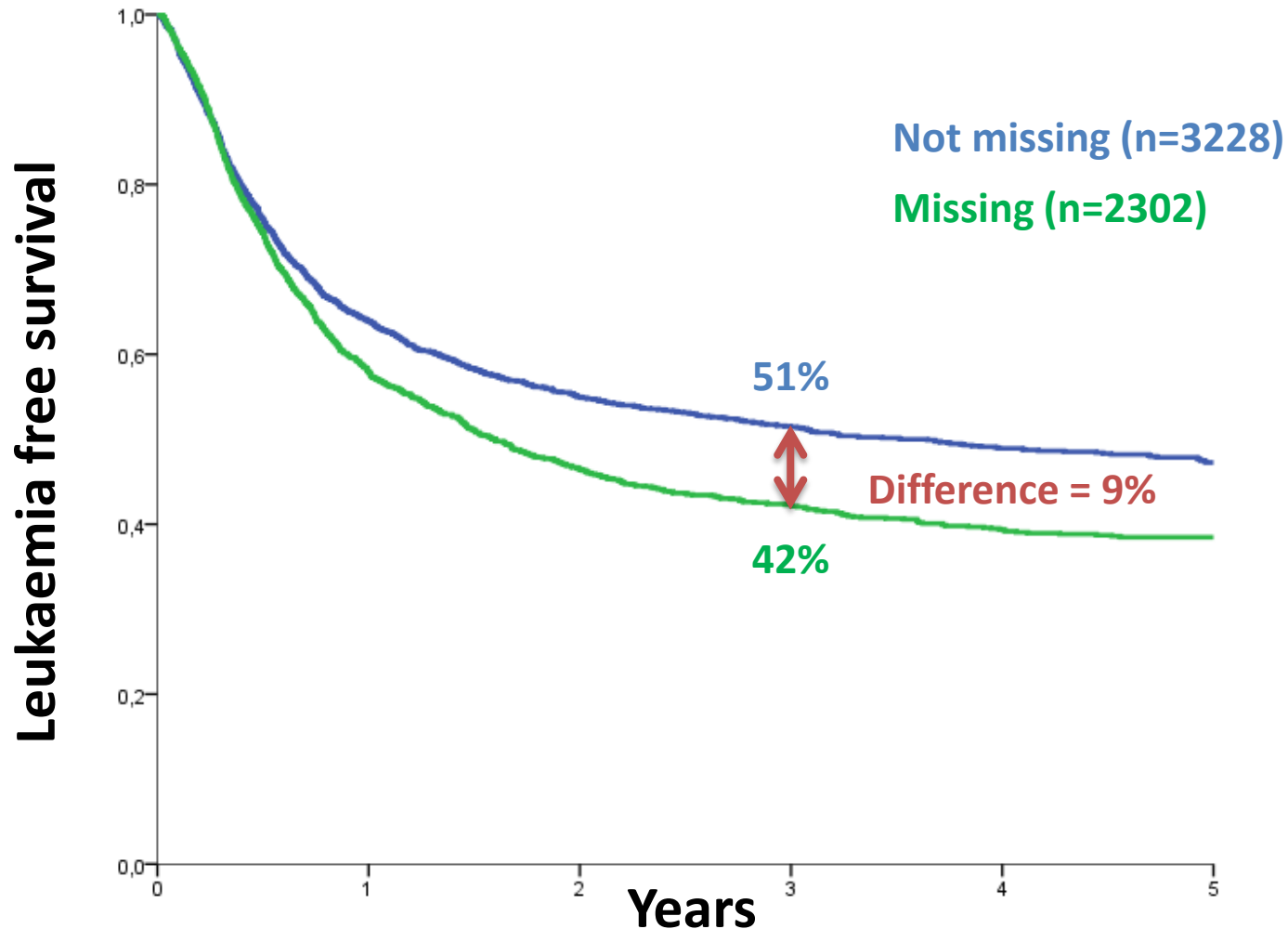
# The Value of Global Outcome Registries : Pros and cons

Question: does CNS involvement adversely affect the outcome of transplant for ALL?



# The Value of Global Outcome Registries : Pros and Cons

## Outcome of myeloablative transplant for AML 2000-2010: influence of cytogenetics



## Data Quality

- **Missing data are not at random => validity of the studies?**
- **Follow-up data are less reported in case of death**
- **MEDB reporting is associated with better outcome compared to MEDA**
- **Reporting “no event” is associated with worse outcome than missing information**

## Data Quality: why are there problems?

- **Complexity and changes over time of the forms, database, software**
- **Lack of communication between designers and users**
- **Lack of data-managers/study coordinators**
- **Heterogeneity in the collection**
  - Different persons -> different knowledge, motivation
  - Follow-up: drop-out, refusal, removal, emigration, follow up only when an event occurs



## **Not so good at answering important questions?**

- **NMA vs RIC vs MAC**
- **Alemtuzamab vs ATG/ALG vs T-replete**
- **Prevention and management of GvHD: 421 studies on-going and registered at [clinicaltrials.gov](https://clinicaltrials.gov)**

## Role of T-cell depletion in VUD for AML CR2

	Numbers per centre			Number of centres	Number of cases
	Min	Max	Med		
Patient numbers	1	16	3	81	356
No ex vivo & in vivo prophylaxis	1	16	3	80	345
No drugs reported	1	16	3	80	343
ATG/ALG	1	7	2	60	146
Alemtuzamab	1	5	2	12	65

# The Value of Global Outcome Registries

- **Retrospective data collection has been invaluable in recording, assessing & optimising transplant practice, & will continue to play a major role in determining transplant activity & methodology**
- **Global debate about how and what data to collect to supplement existing registry data in an affordable manner**
- **The future lies in multicentre prospective studies**
- **In places where multicentre studies are difficult, collaboration on protocols might be a valuable start**

# The Value of Global Outcome Registries

## What's in it for you?

- **Being part of a global community**
- **Knowing that you are treating your patients as well as you can**
- **Participating in retrospective and prospective studies**
- **Satisfying accreditation and payers requirements**
- **Influencing the future**



*The European Group for Blood and Marrow Transplantation*