


Future Trends in Hematopoietic Cell Transplantation (HCT)

A decorative graphic consisting of several overlapping, curved blue lines and small blue dots scattered across the lower half of the slide.

Dennis Confer, MD
NMDP Chief Medical Officer
November 1, 2007

Current and Future Trends

- Registries of adult donors and cord blood units are growing larger
- Transplant numbers are increasing
- Patient populations are changing
- Transplant survival is improving

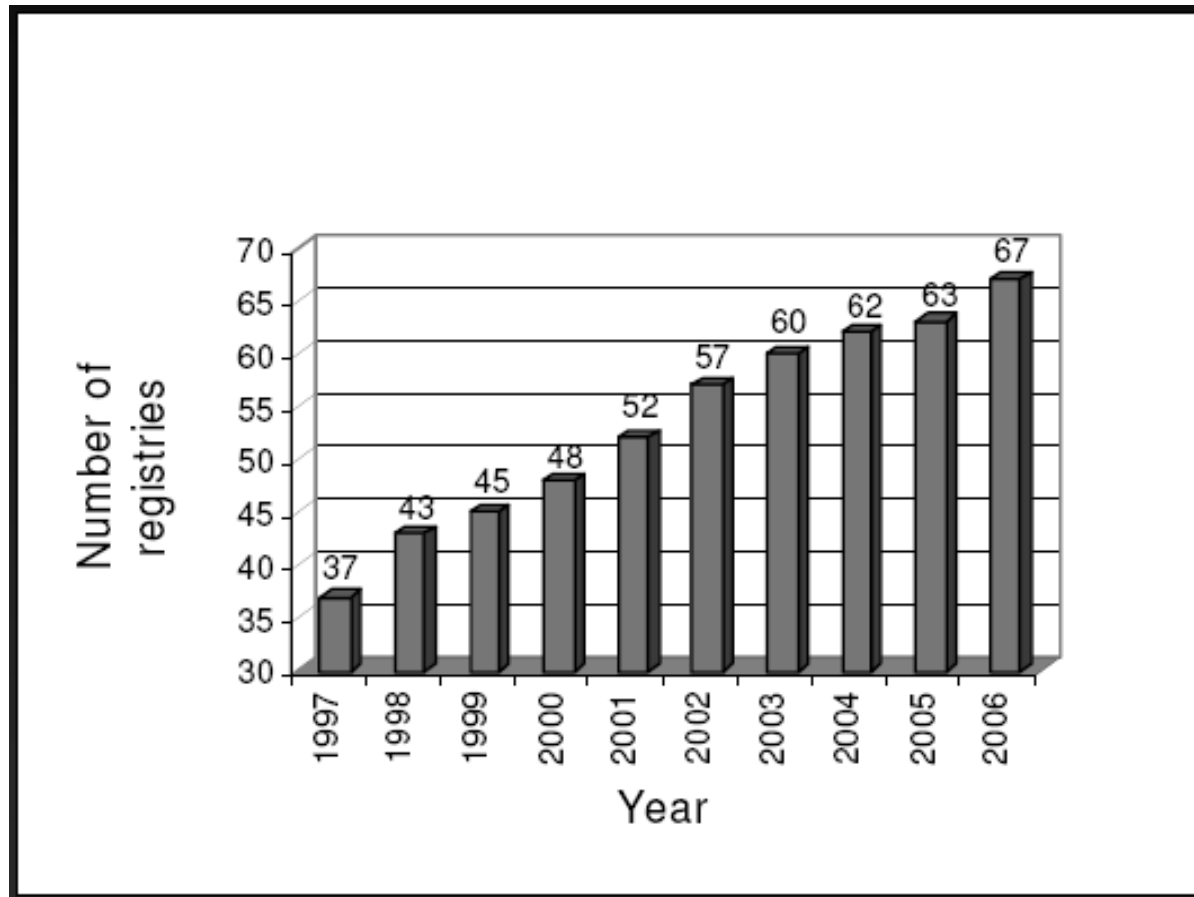
World Marrow Donor Association - WMDA

- Association of adult donor registries and public cord blood banks engaged in international delivery of HCs
- Annual member survey assesses worldwide donor numbers and transplantation activity
- www.worldmarrow.org

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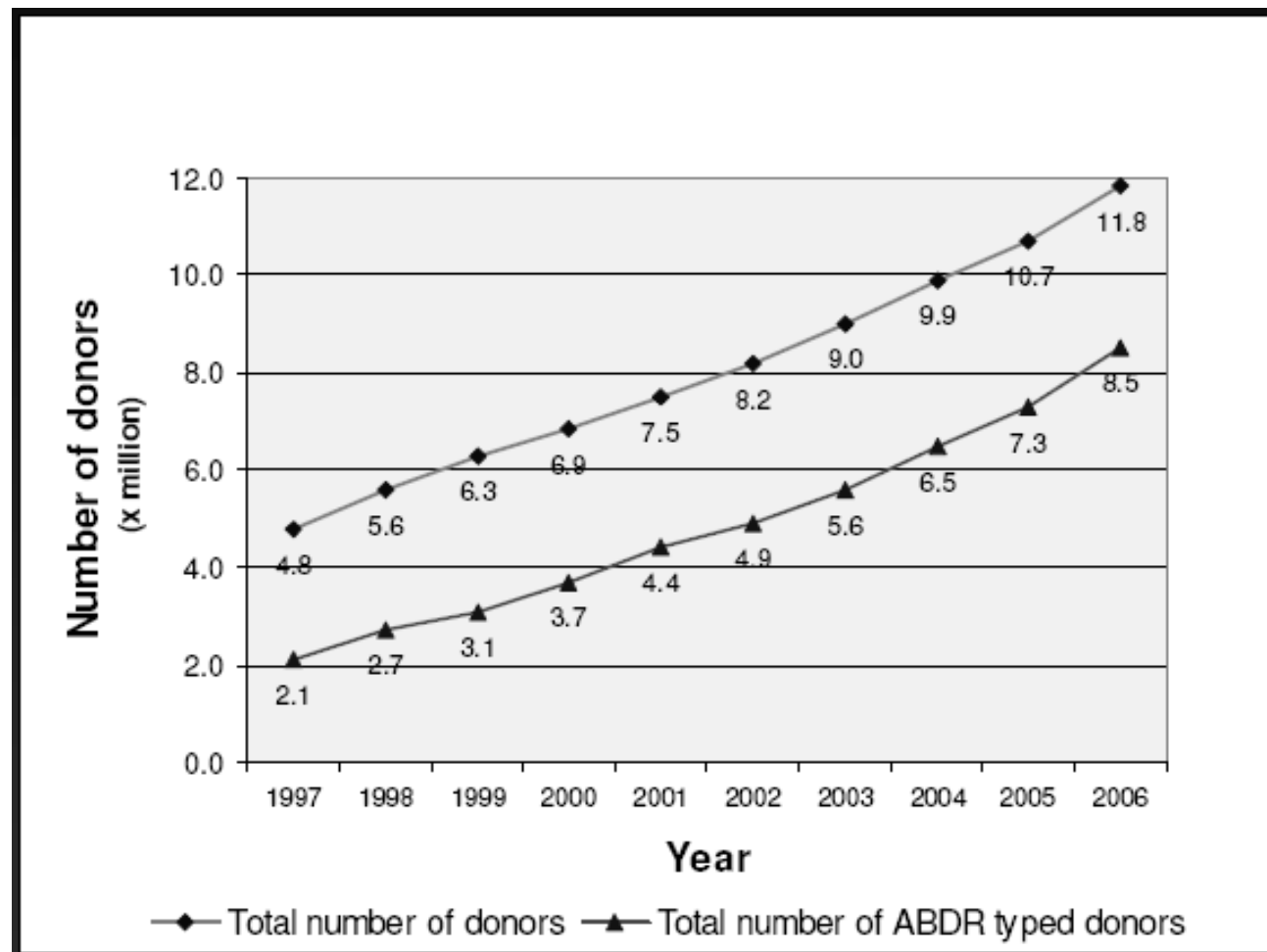
Adult Donor Registries Reporting to WMDA



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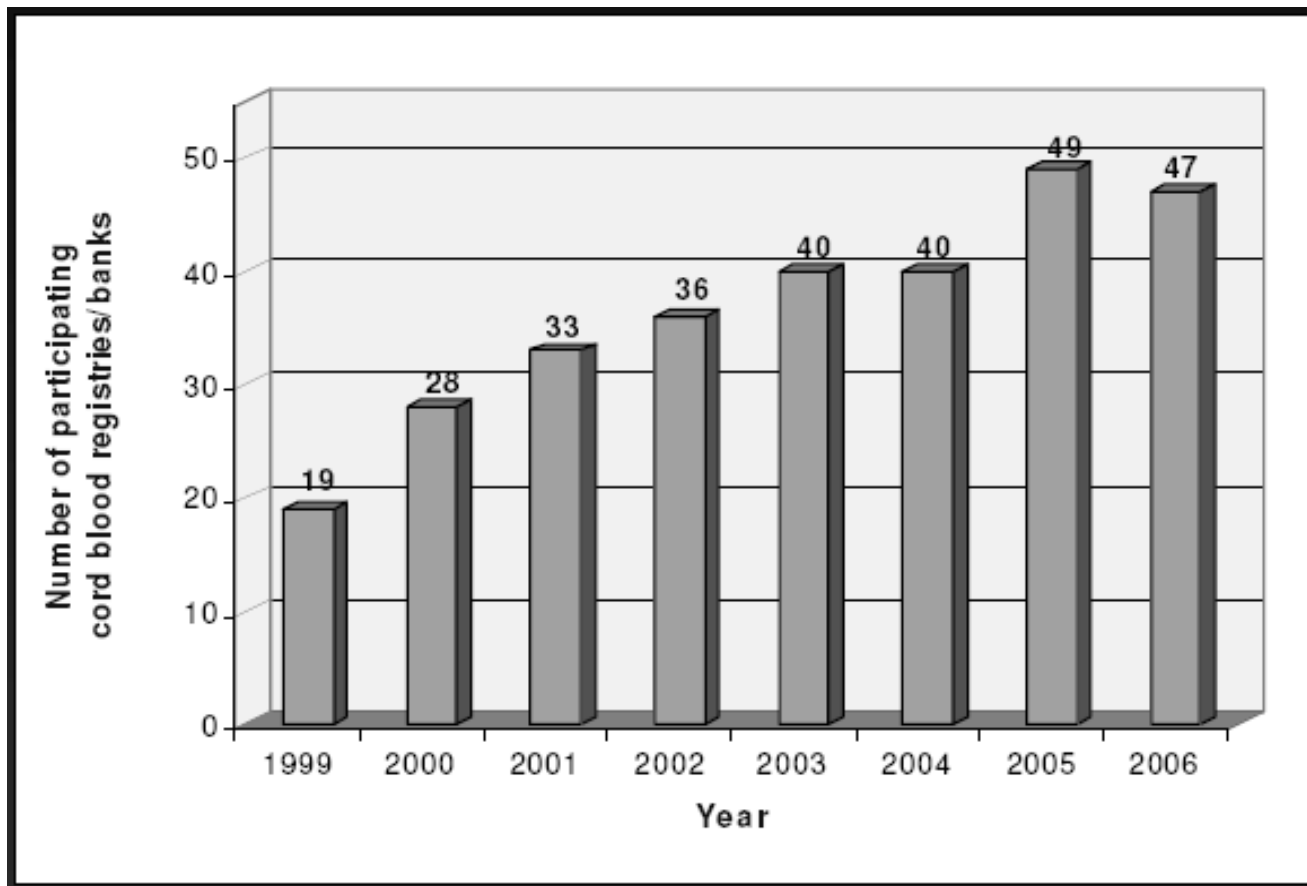
Adult Donors Worldwide



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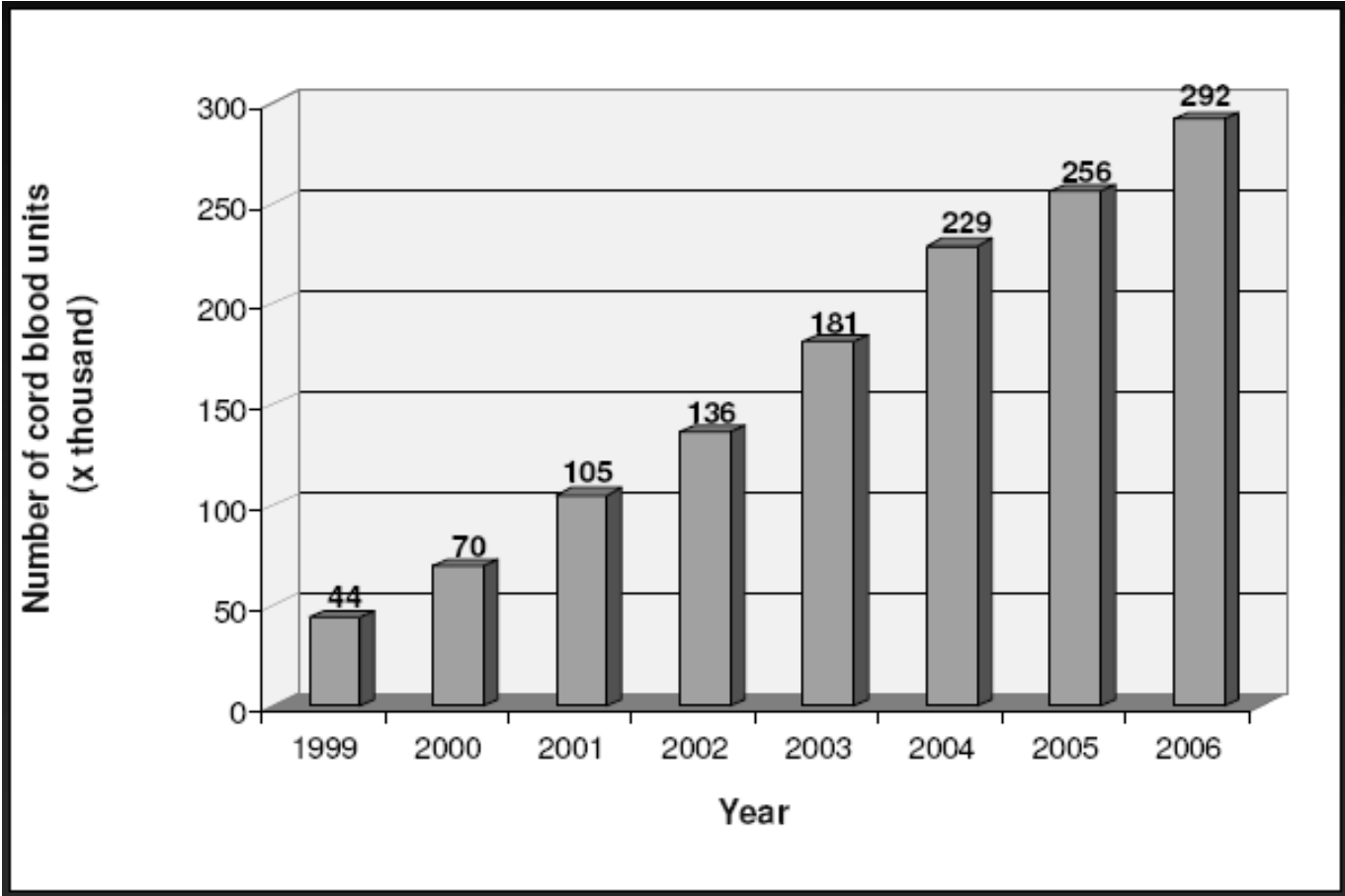
Cord Blood Banks Reporting to WMDA



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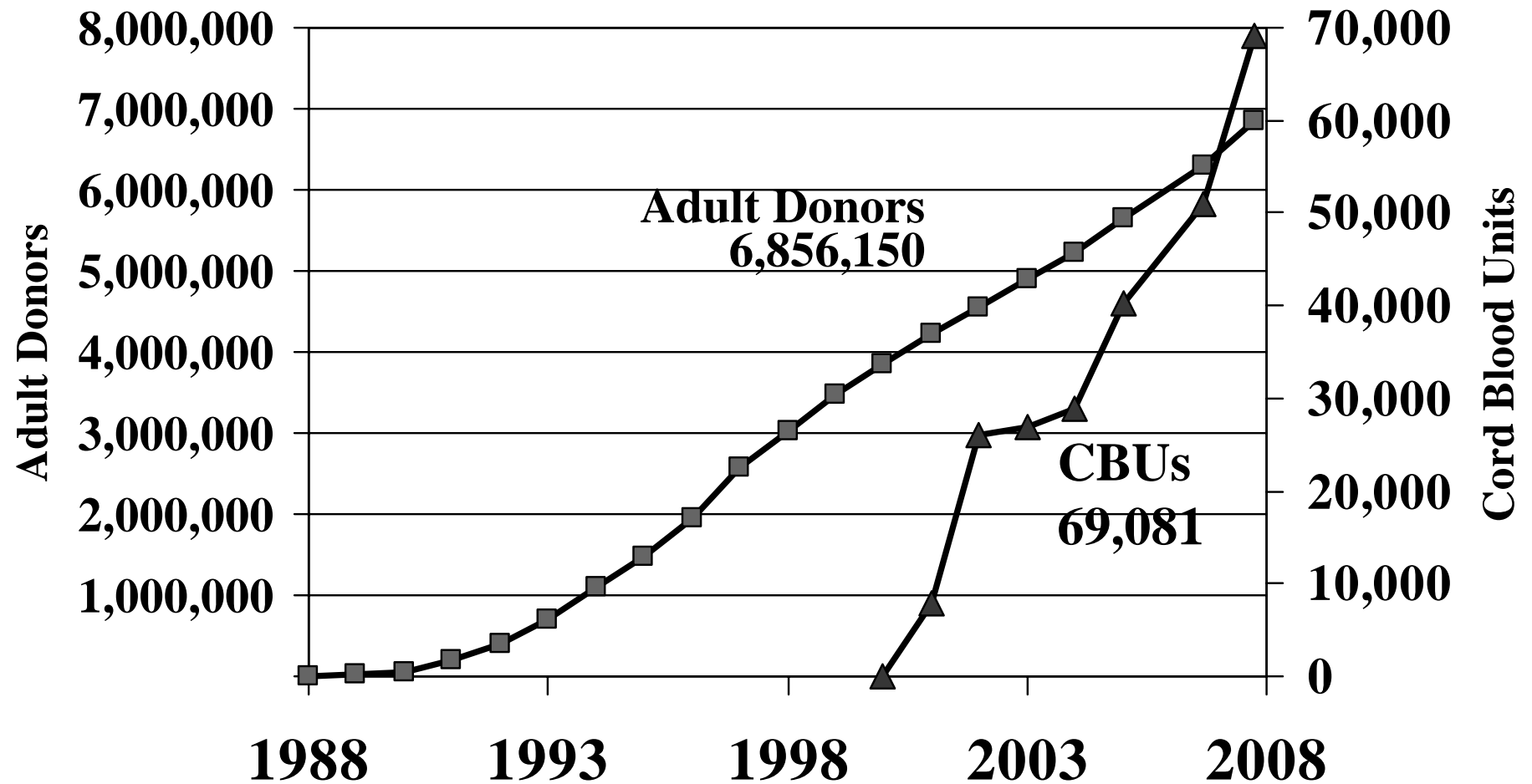
Cord Blood Units Worldwide



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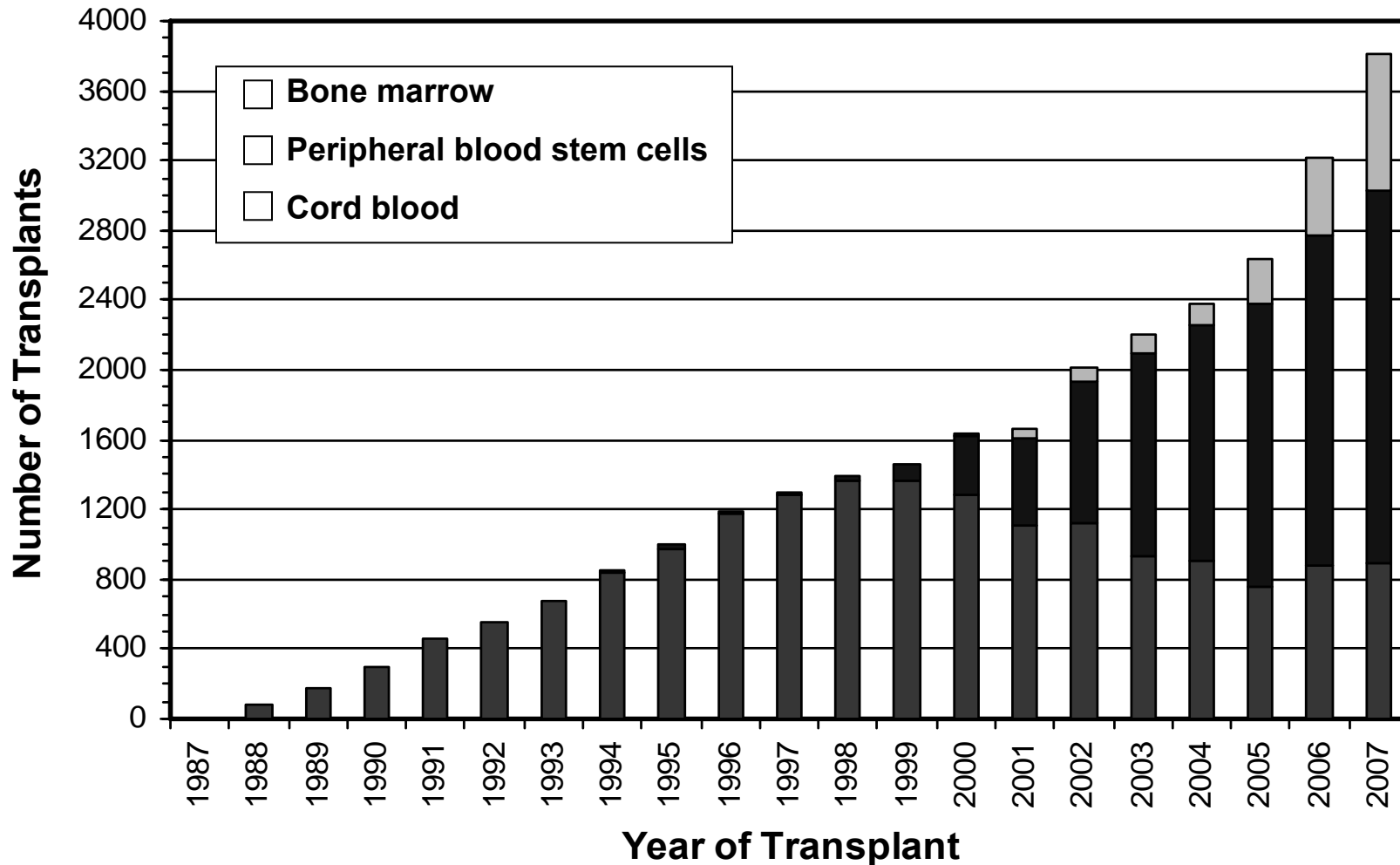
National Marrow Donor Program Adult Donors & CBUs– Sept 30, 2007



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NMDP Transplants Facilitated

by Fiscal Year 1987–2007

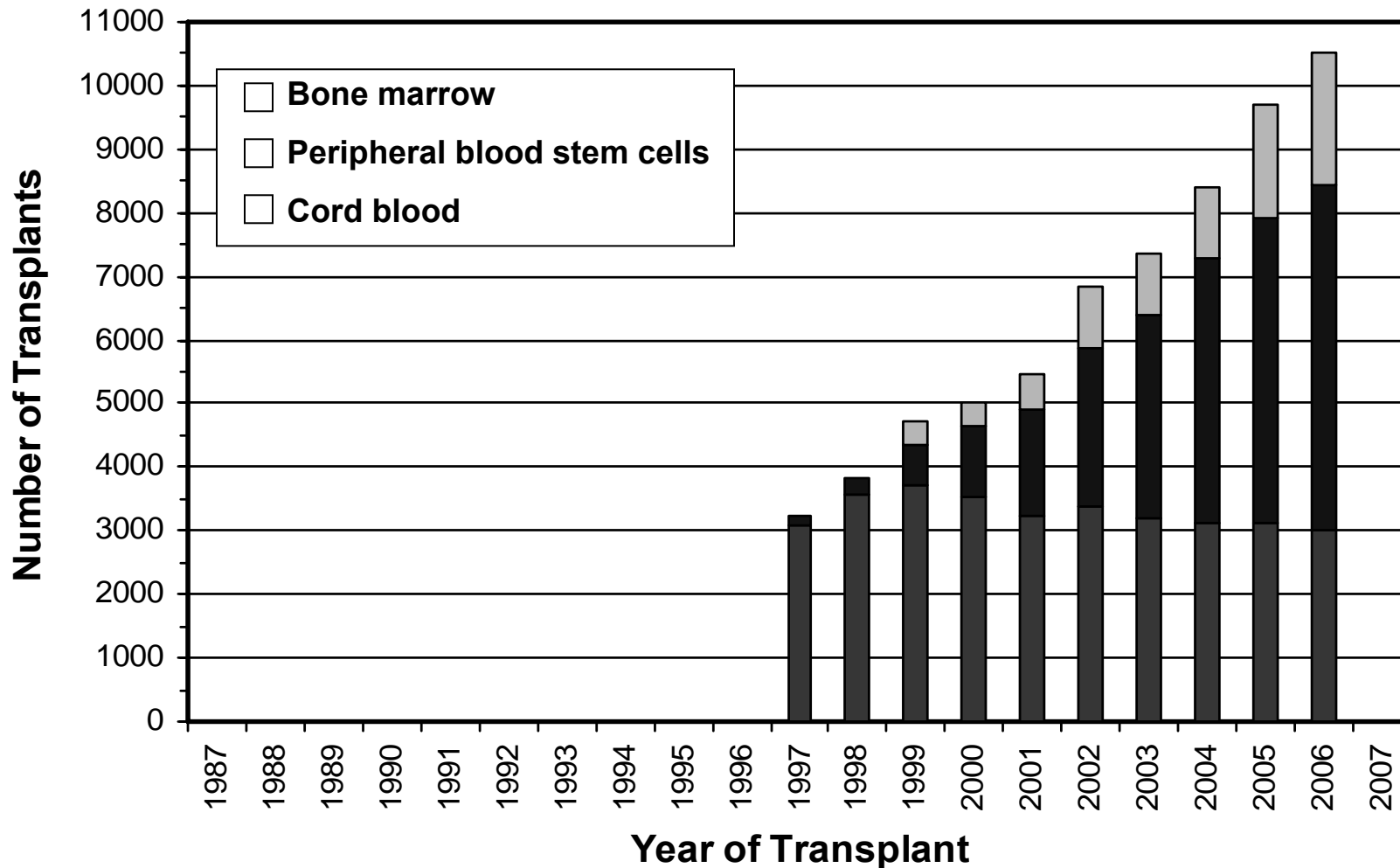


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Worldwide Transplants Facilitated

by Calendar Year 1997–2006

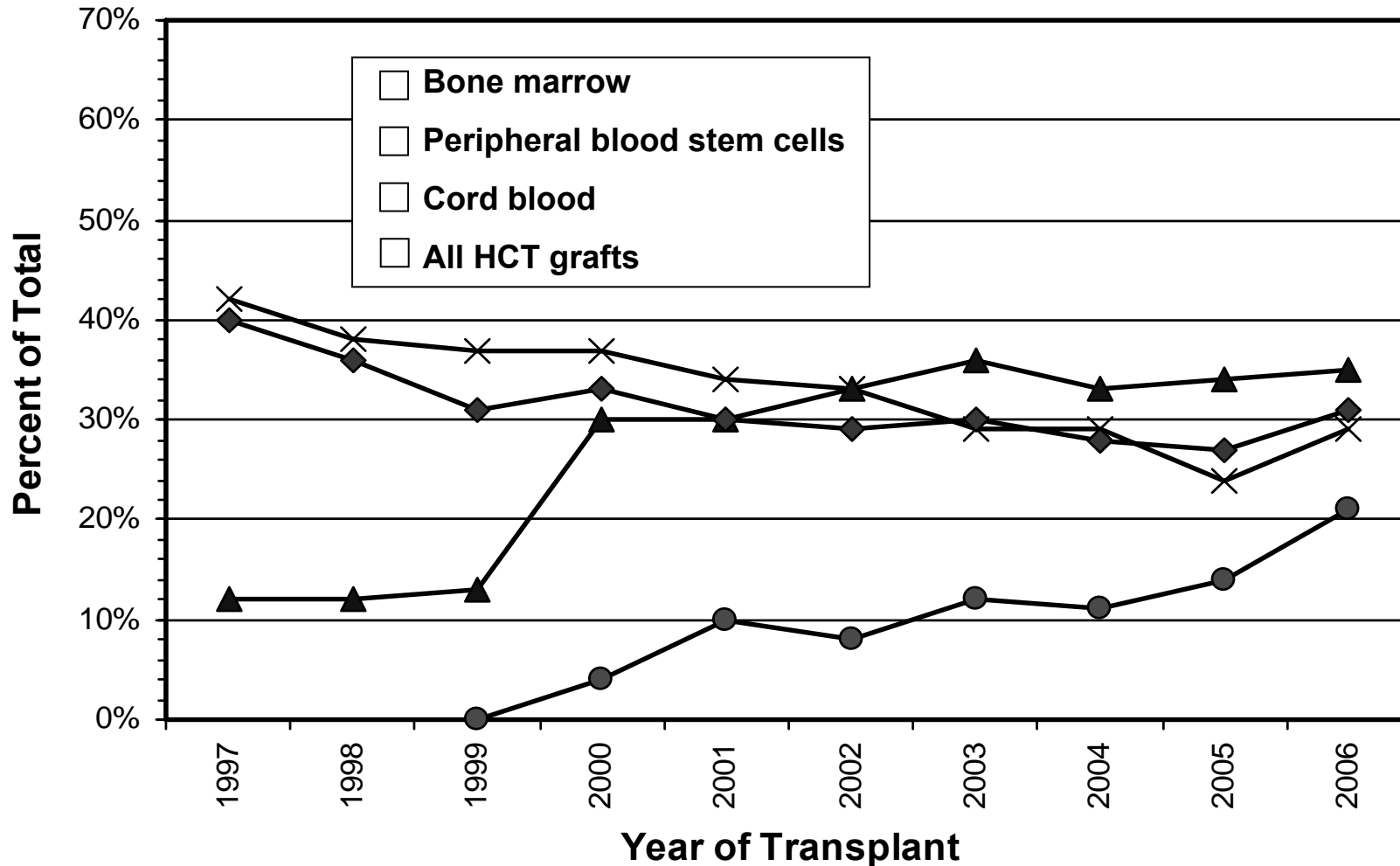


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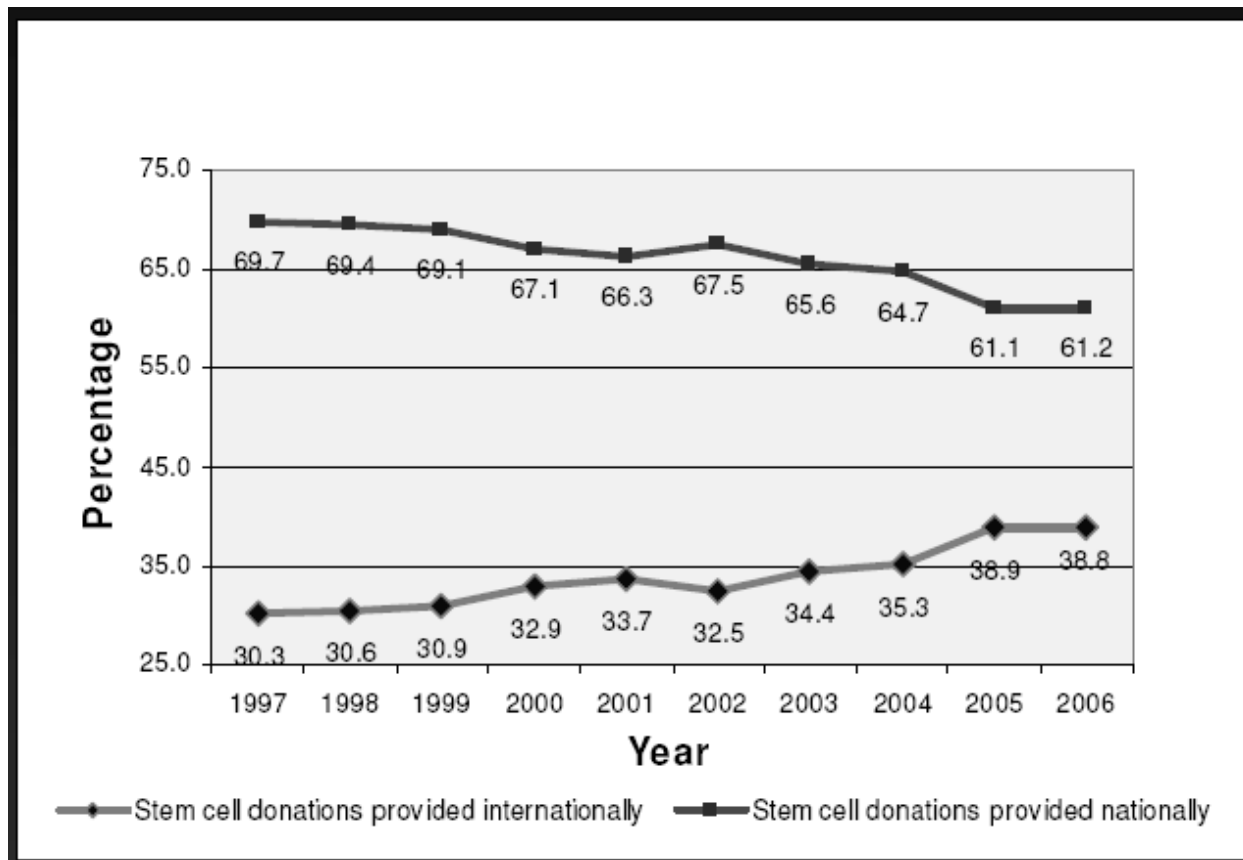
NMDP Grafts as a Percent of the WMDA Total

by Calendar Year 1997–2006



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Adult Donor Registries Reporting to WMDA



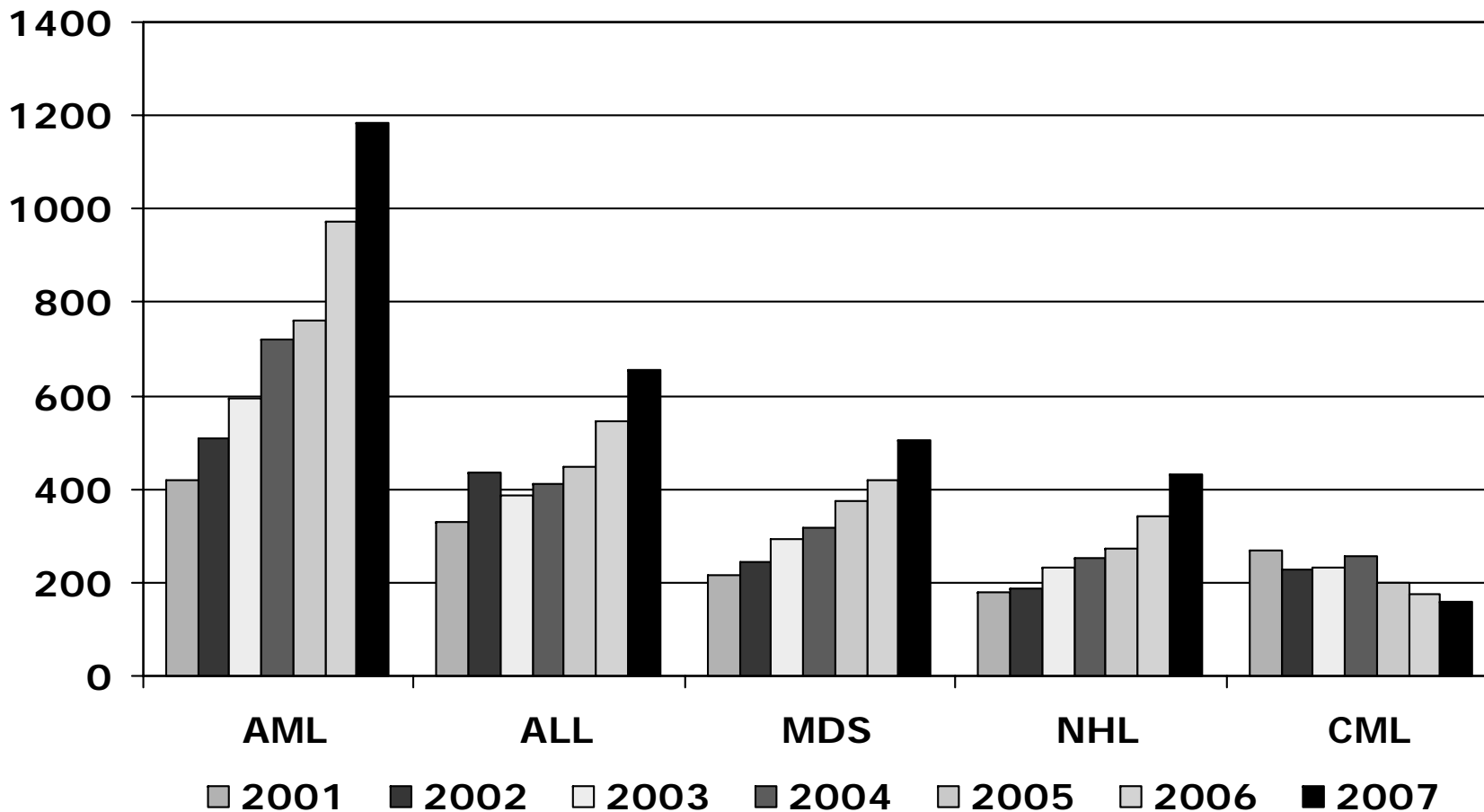
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Current and Future Trends

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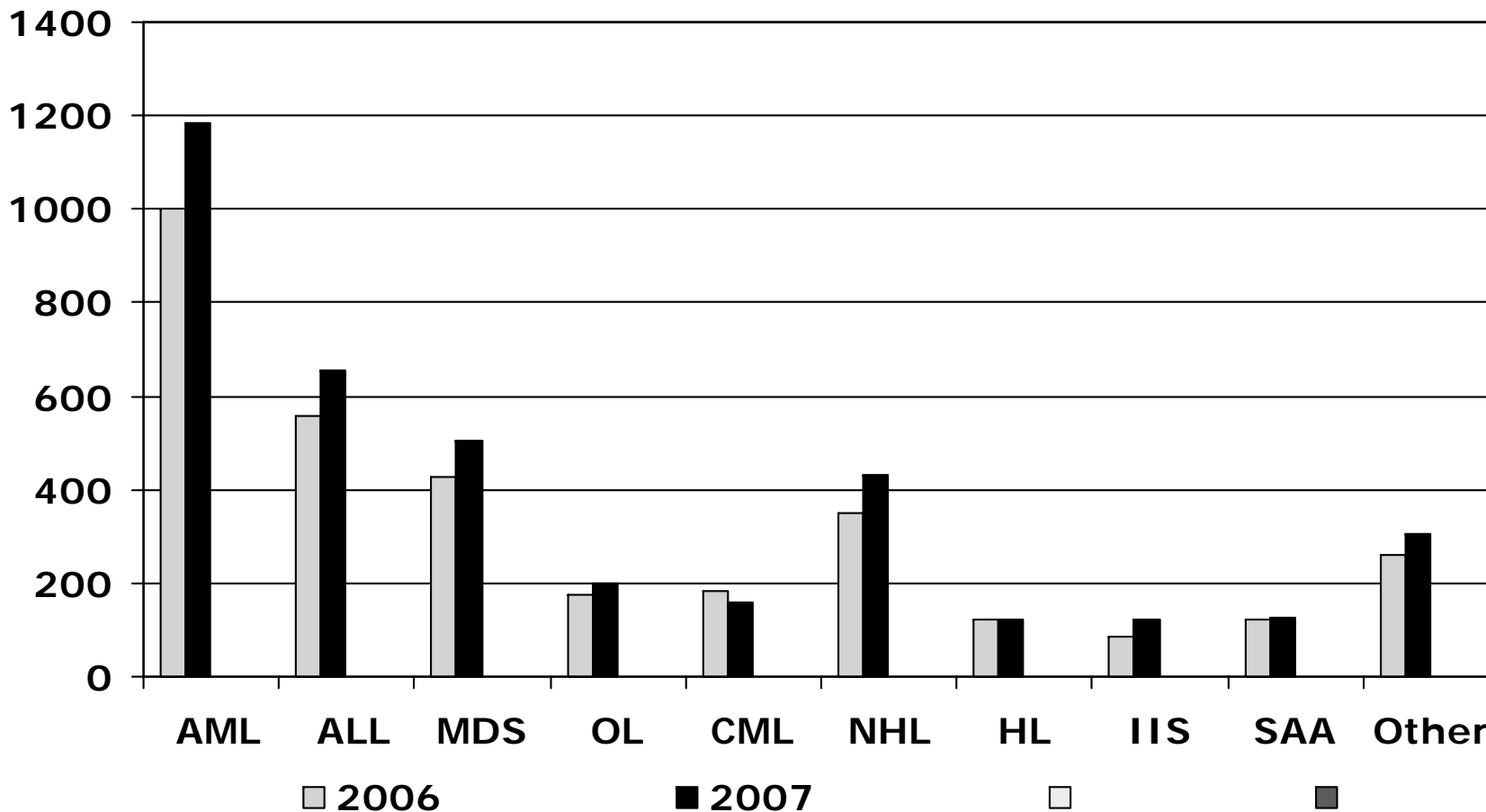
NMDP Transplant Recipients by Diagnosis



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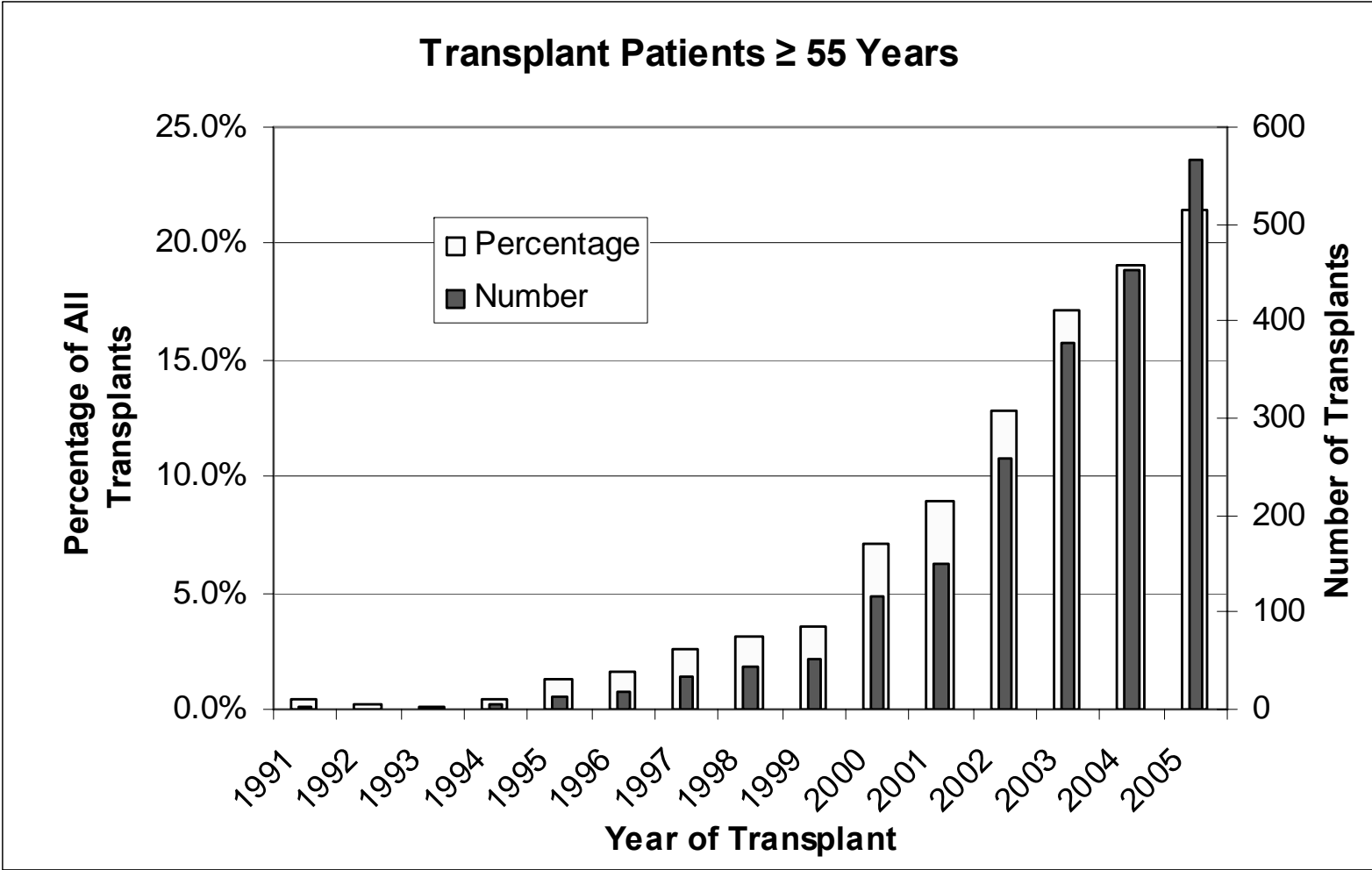
NMDP Transplant Recipients by Diagnosis



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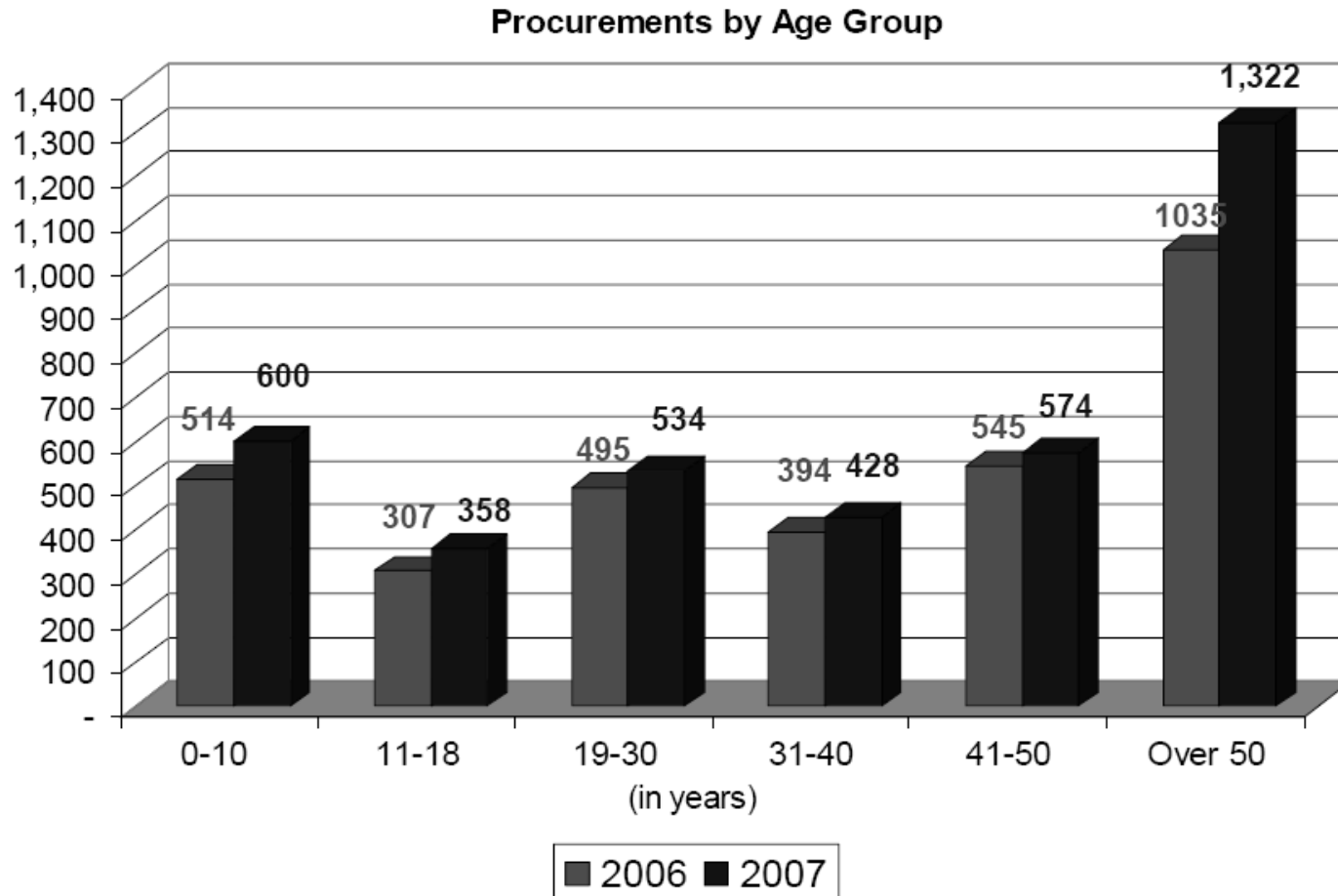
More Transplant Recipients are Older



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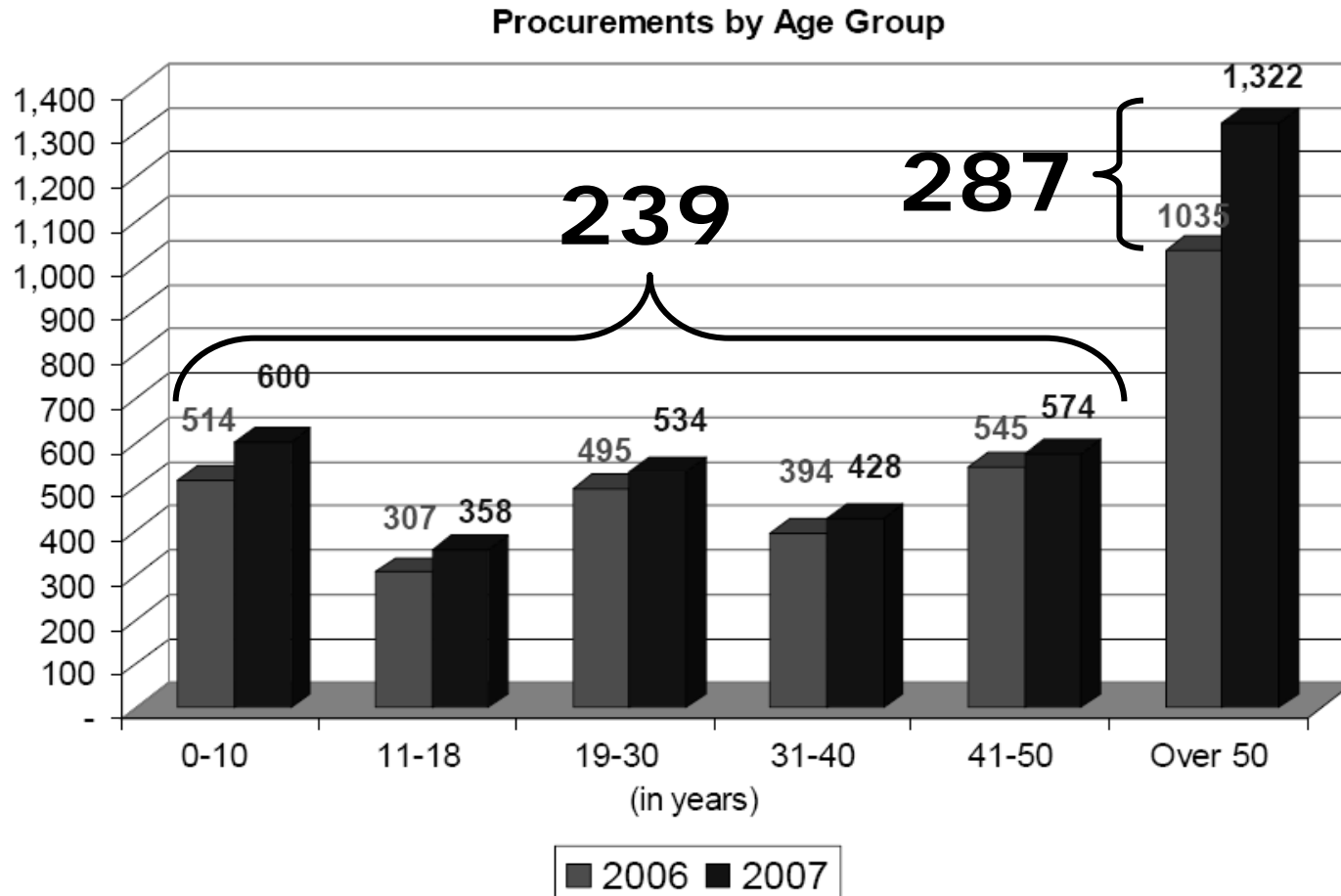
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Transplant Recipients by Age Group FY 2006 and FY 2007



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Transplant Recipients > 50 years Account for Most of the Growth



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Improving Survival: All Adults with Malignant Diseases

- These curves show statistically significant improvements in overall survival comparing 4 time periods with similar numbers of patients
 - 1987 – 94: n = 1674
 - 1995 – 97: n = 1958
 - 1998 – 2000: n = 2185
 - 2001 – 2004: n = 1811

Improving Survival: Adults with High-Risk Malignant Diseases

- Similar results with a more dramatic impact of recent years are seen restricting the analysis to only High-risk adults
 - 1987 – 94: n = 807
 - 1995 – 97: n = 910
 - 1998 – 2000: n = 1011
 - 2001 – 2004: n = 948

Improving Survival: Adults Aged 18 – 54 with Malignant Diseases

- Excluding patients over age 54 modestly improves survival compared the first set of curves, but note the rise in older patient numbers
 - 1987 – 94: n = 1666, excluded 8
 - 1995 – 97: n = 1897, excluded 61
 - 1998 – 2000: n = 2057, excluded 128
 - 2001 – 2004: n = 1550, excluded 261

Improving Survival: Adults with Non-Malignant Diseases

- These curves show statistically significant improvements in overall survival comparing 4 time periods with similar, but smaller numbers of patients with non-malignant diseases
 - 1987 – 94: n = 81
 - 1995 – 97: n = 56
 - 1998 – 2000: n = 70
 - 2001 – 2004: n = 82

Improving Survival: Pediatrics with Malignant Diseases

- These curves show statistically significant improvements in overall survival comparing 4 time periods with pediatric patients
 - 1987 – 94: n = 646
 - 1995 – 97: n = 666
 - 1998 – 2000: n = 638
 - 2001 – 2004: n = 657

Improving Survival: Pediatrics with Non-Malignant Diseases

- These curves show statistically significant improvements in overall survival comparing 4 time periods of pediatric patients with non-malignant diseases
 - 1987 – 94: n = 225
 - 1995 – 97: n = 228
 - 1998 – 2000: n = 234
 - 2001 – 2004: n = 253

Further Evidence of Improving Survival

- Center-specific analysis – Evaluates one-year survival at all US transplant centers
- Employs a rolling 5-year window

Report Year	Period	1-Year Survival
2003	1996-2001	42.2%
2006	2000-2004	48.8%
2007	2001-2005	51.5%

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Monitoring Survival over Time - PART: Program Assessment Rating Tool

- Initiative of the President's Office of Management and Budget (OMB)
- Evaluates government programs on multiple measures
- Must include a Health Outcome Measure
- We proposed:
 - Aggregate 1-year survival for a defined subset of patients evaluated over time

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PART: Program Assessment and Rating Tool

- Restricted to patients <50 years age
- Myeloablative regimens only
- Acute leukemias in any remission
- Chronic Myelogenous Leukemia
- MDS – RA or RARS only
- First analysis completed in May 2006
 - 5,432 recipients through December 31, 2003

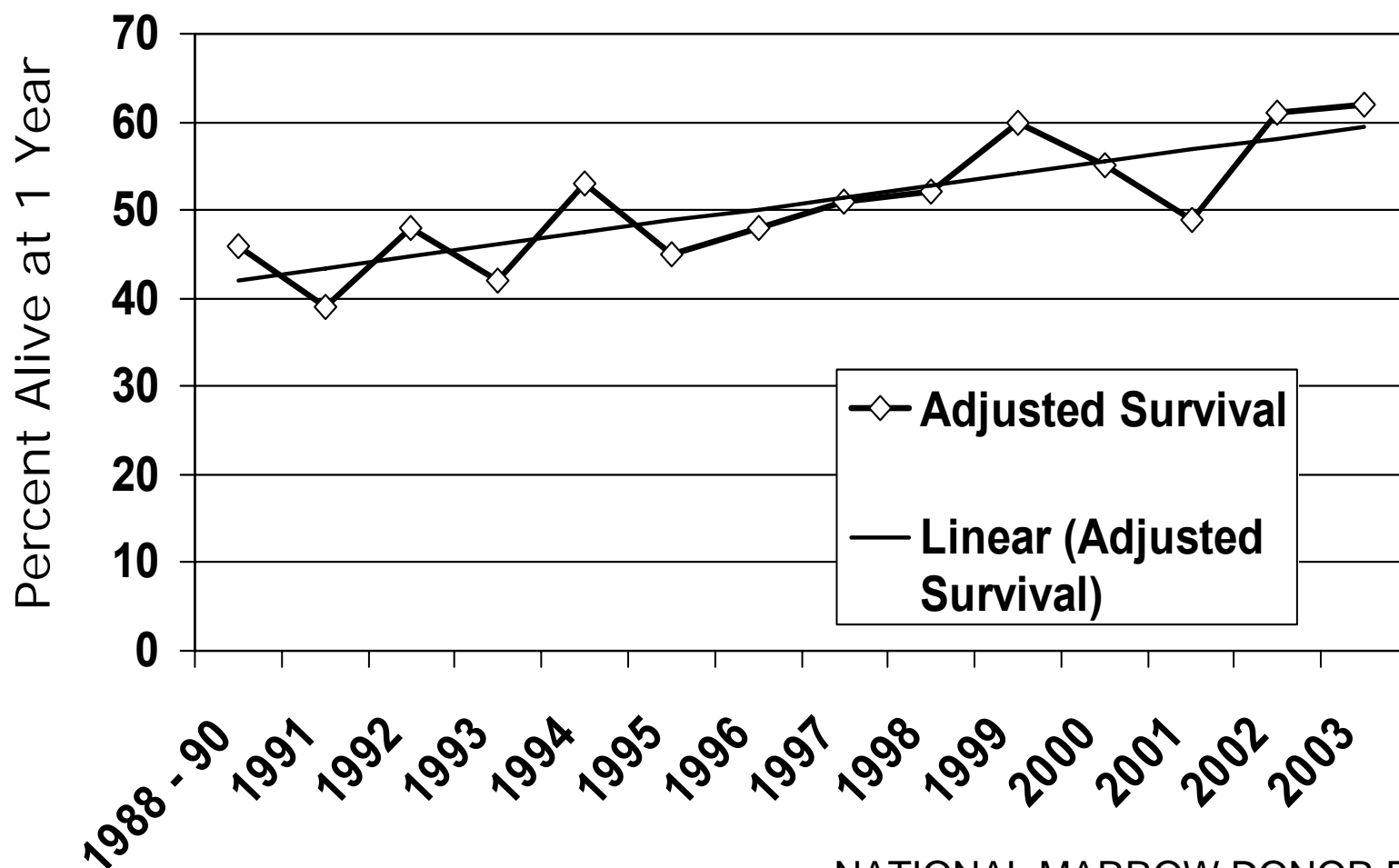
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Factors Considered in the Modeling for the PART Analysis

- Recipient age
- Recipient sex
- Recipient race
- Recipient CMV status
- Recipient BMI
- Disease and Stage
- Time from Dx to Tx
- Disease Risk
- Karnofsky/Lansky
- Coexisting disease

Adjusted 1-year Survivals over Time: Part Analysis – 1988 - 2003



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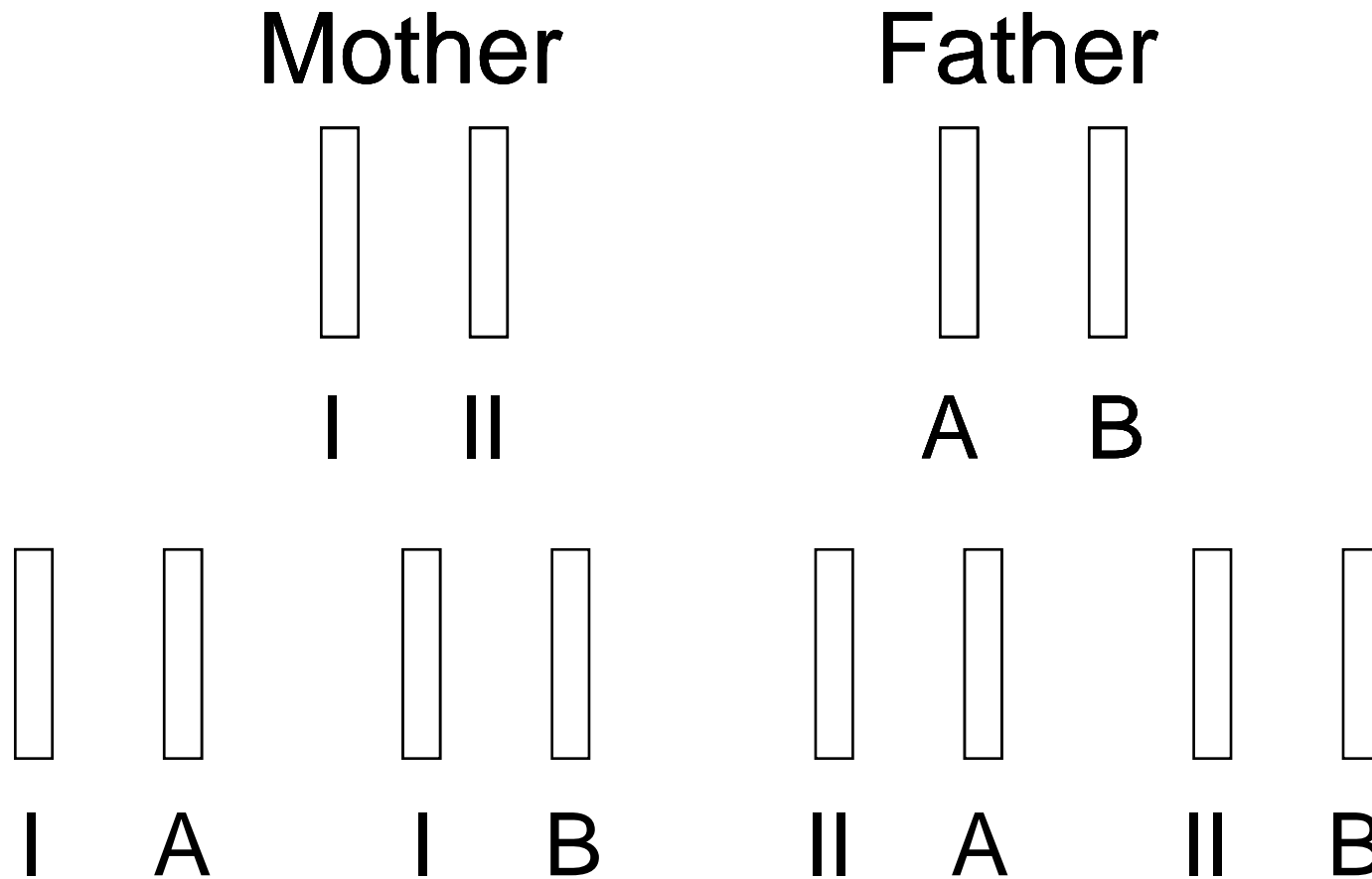
Current and Future Trends

- Registries of adult donors and cord blood units are growing larger
- Transplant numbers are increasing
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How Many Transplants Should We Be Doing?

- Approaches to an Answer:
 - Infer from the Matched Sibling Transplants
 - Infer from the Search Activity
 - Infer from knowledge of diseases
- These numbers are U.S.A. only!

The Likelihood of a Match Between Two Siblings is 25%



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Probability of a Sibling Match

- Either you do or you don't
 - Probability of a match = 1 – Probability of no match
- One sibling
 - Probability of a match = 1 – 0.75 = 25%
- Two siblings
 - Probability of a match = 1 – (0.75*0.75) = 44%
- Six siblings
 - Probability of a match = 1 – (0.75)⁶ = 82%
- 1.3 siblings
 - Probability of a match = 1 – (0.75)^{1.3} = 31%

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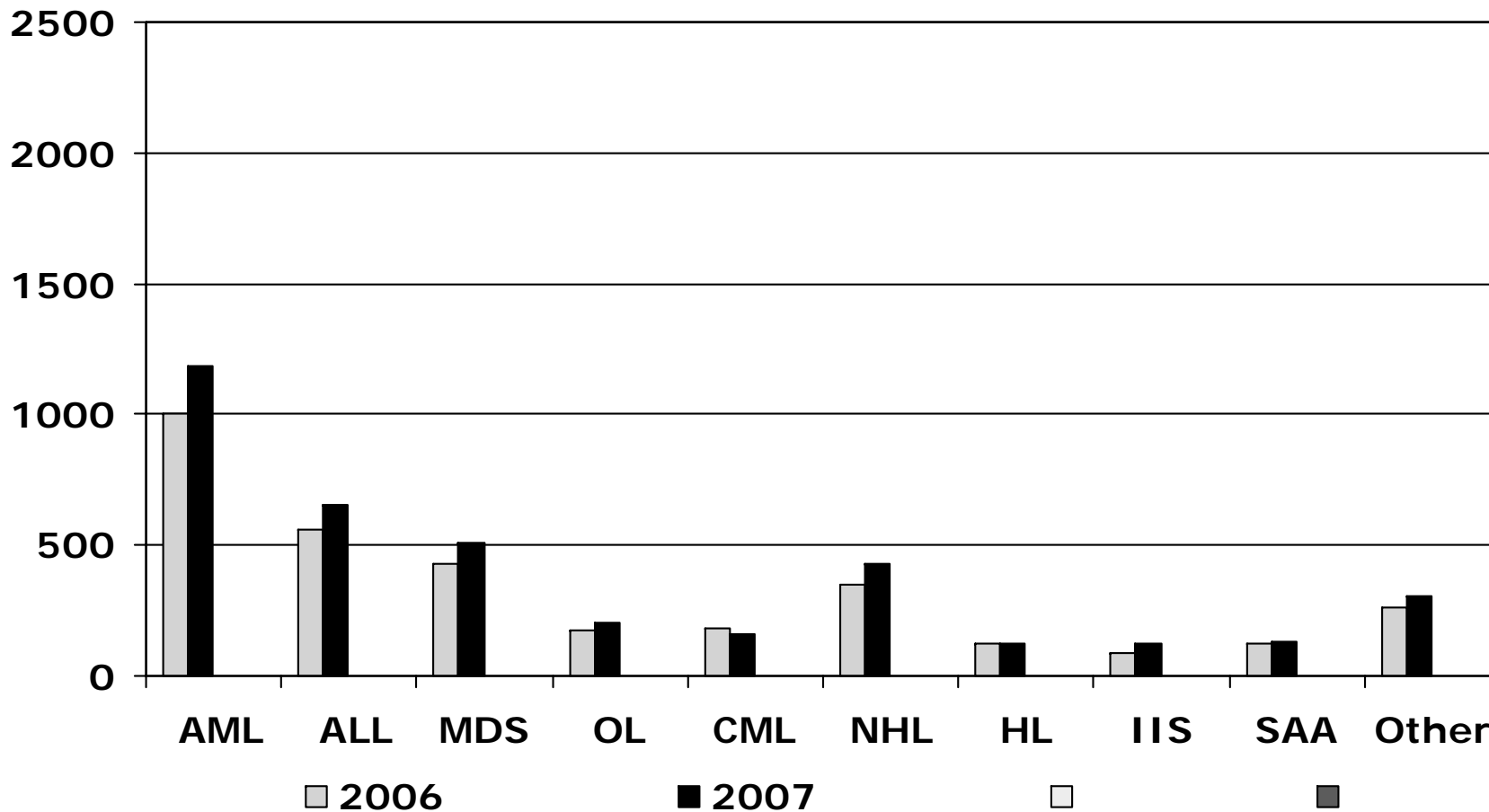
How Many Transplants Should We Be Doing?

- Infer from the Matched Sibling Transplants
 - CIBMTR estimates 3,500 matched sibling transplants annually in the U.S.
 - $3,500/0.3 = 11,667$ is the “Total Need”
 - $11,667 - 3,500 = 8,167$ are candidates for unrelated transplants

How Many Transplants Should We Be Doing?

- Infer from the Unrelated Donor Search Activity
 - NMDP received 7,249 U.S. preliminary searches in the past 12 months

NMDP Transplant Recipients by Diagnosis



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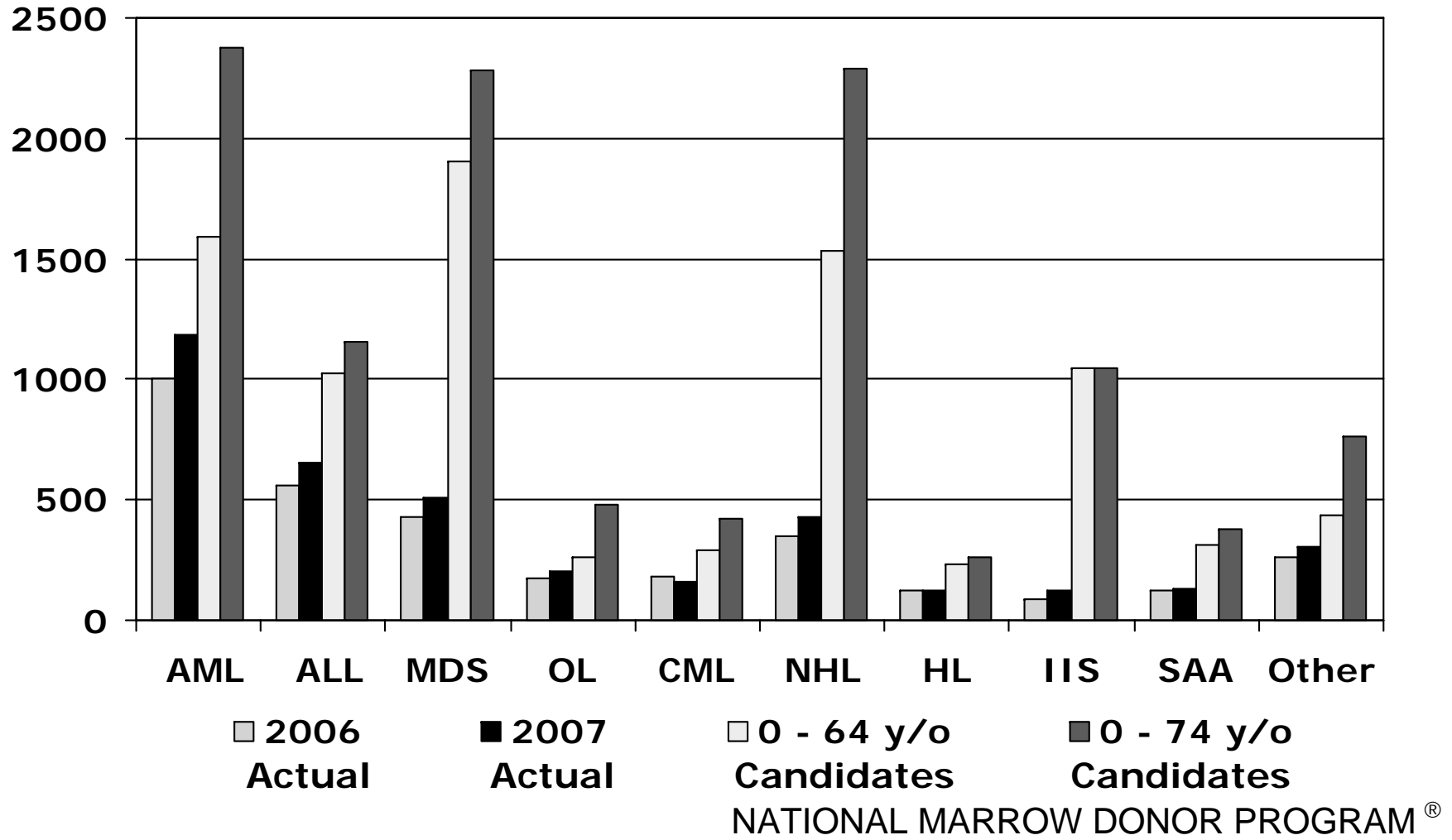
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How Many Transplants Should We Be Doing?

- Infer from the Knowledge of Diseases
 - Estimate the incidence of disease by age group
 - Calculate the number of new cases annually
 - Estimate those who are candidates for transplantation
 - Partition between sibling and unrelated

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NMDP Transplant Recipients by Diagnosis and Estimated Candidates



How Many Transplants Should We Be Doing?

- Approaches to an Answer:
 - Infer from the Matched Sibling Transplants = 8,167
 - Infer from the Search Activity = 7,249
 - Infer from knowledge of diseases = 8,500 – 10,000

How Many Transplants Should We Be Doing?

- Approaches to an Answer:
 - Infer from the Matched Sibling Transplants = 8,167
 - Infer from the Search Activity = 7,249
 - Infer from knowledge of diseases = 8,500 – 10,000
- None of these considers emerging indications

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Future Trends

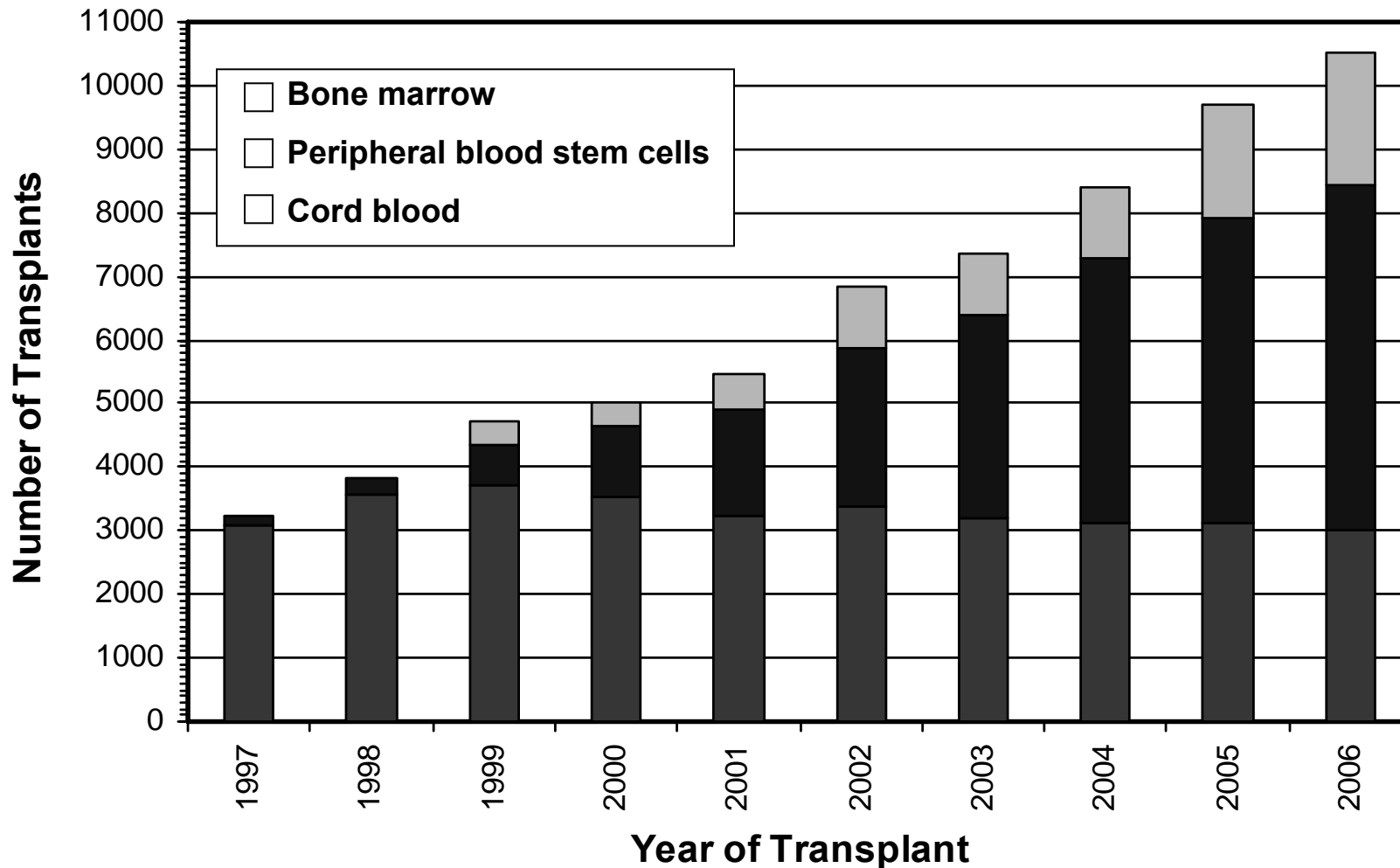
- Registries of adult donors and cord blood units continue to grow
- Transplant numbers will increase, approaching 10,000 in the U.S. alone
- Patient populations will continue to evolve
- Successful transplantation will be the expectation

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Worldwide Transplants Facilitated

by Calendar Year 1997–2006



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