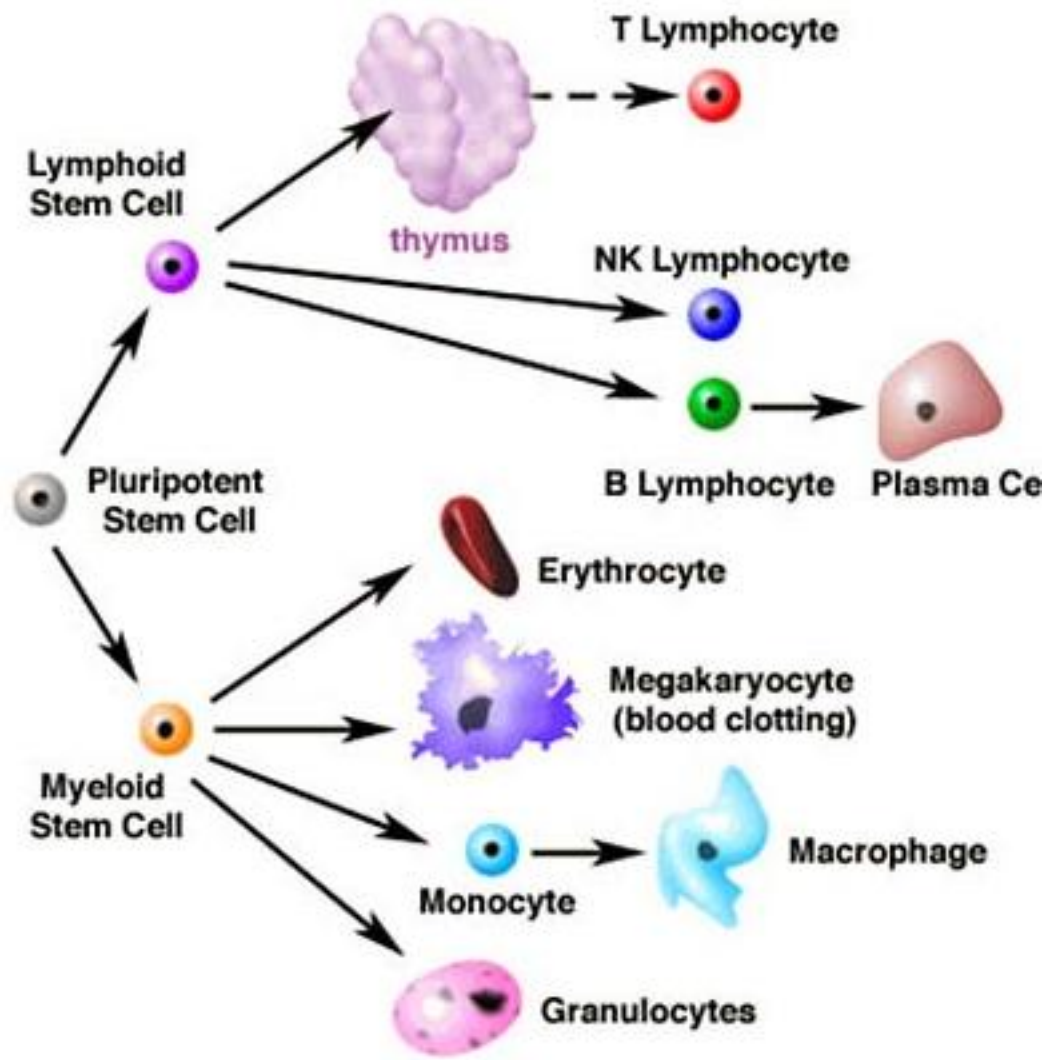


Barriers to Access for Hematopoietic Transplantation

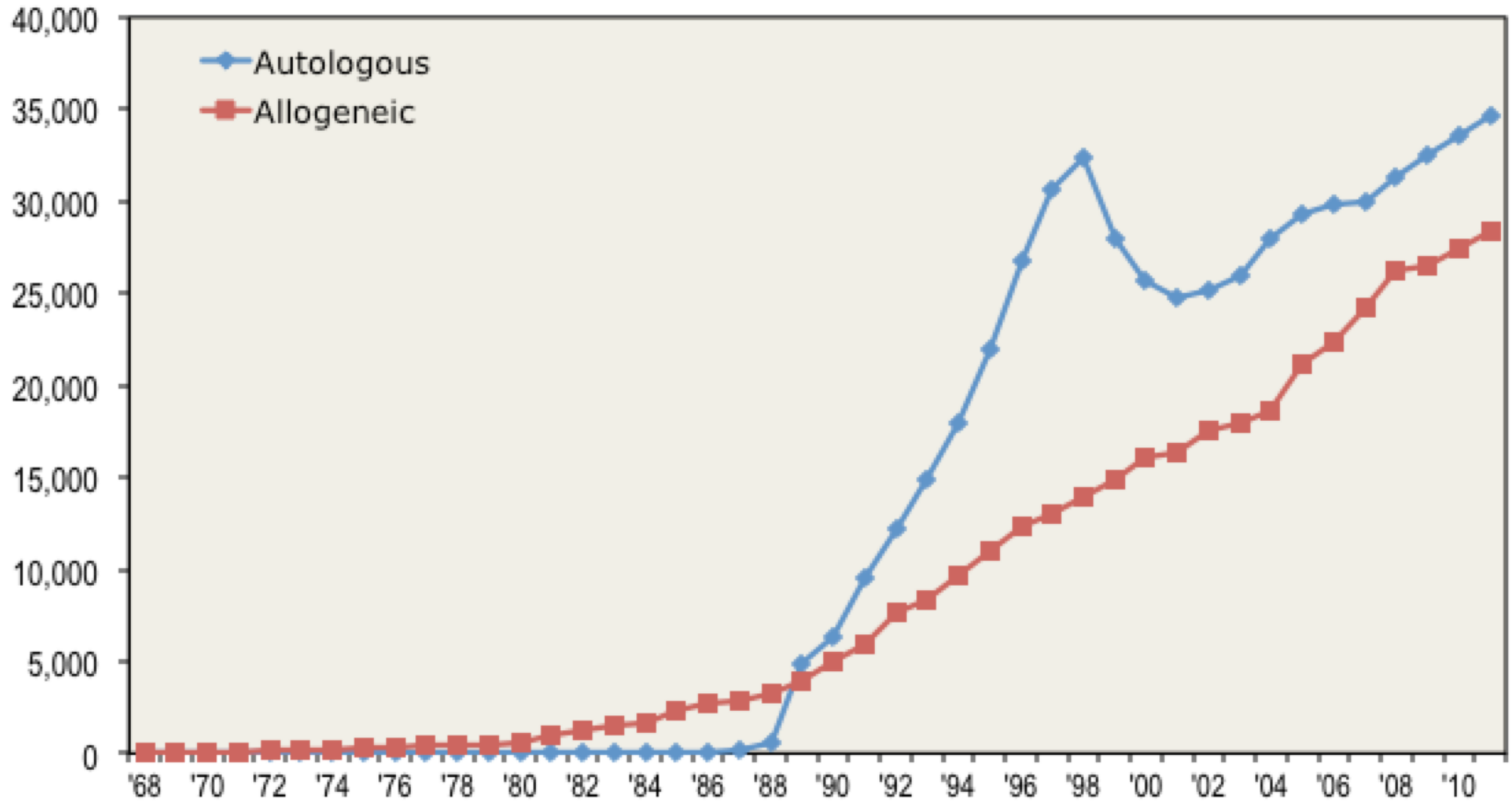
Richard Champlin, MD



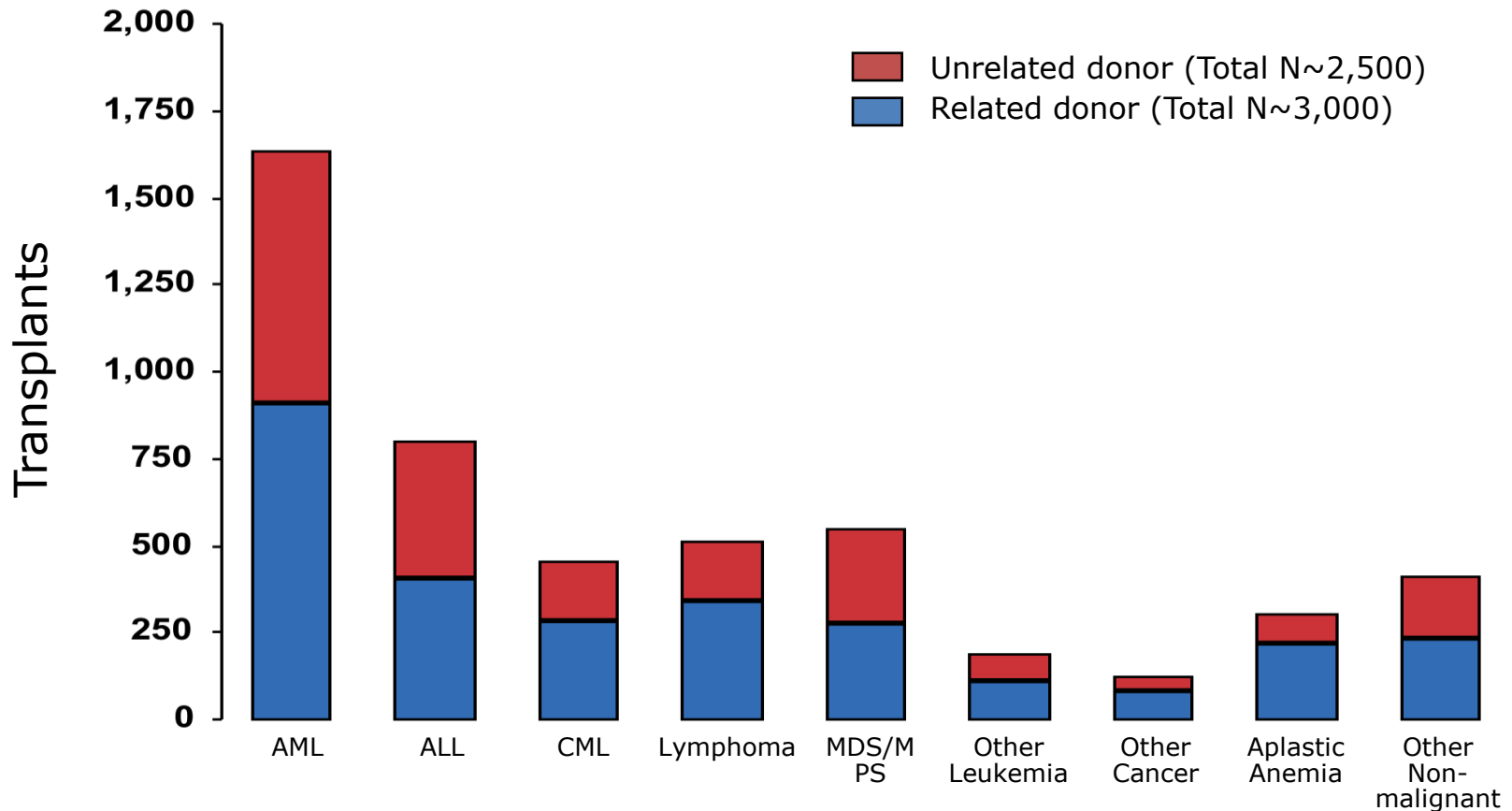
Current State of HSCT

- HSCT is an effective treatment for a broad range of hematologic, immune, metabolic and neoplastic diseases. HSCT has been a major medical advance. ED Thomas awarded Nobel Prize in 1990.
- HSCT replaces recipient blood and immune system with donor cells. For nonmalignant diseases, defective hematopoietic and immune cells are replaced by normal cells; this offers definitive treatment for a broad range of hereditary and acquired diseases.
- For malignancies, benefit related to high dose therapy and for allogeneic transplants, the immune graft-vs-malignancy effects
- Rapidly advancing area of clinical research. HSCT offers a platform for cellular engineering with genetic modification of stem cells, lymphocytes and other hematopoietic cells

Growth in HSCT- CIBMTR



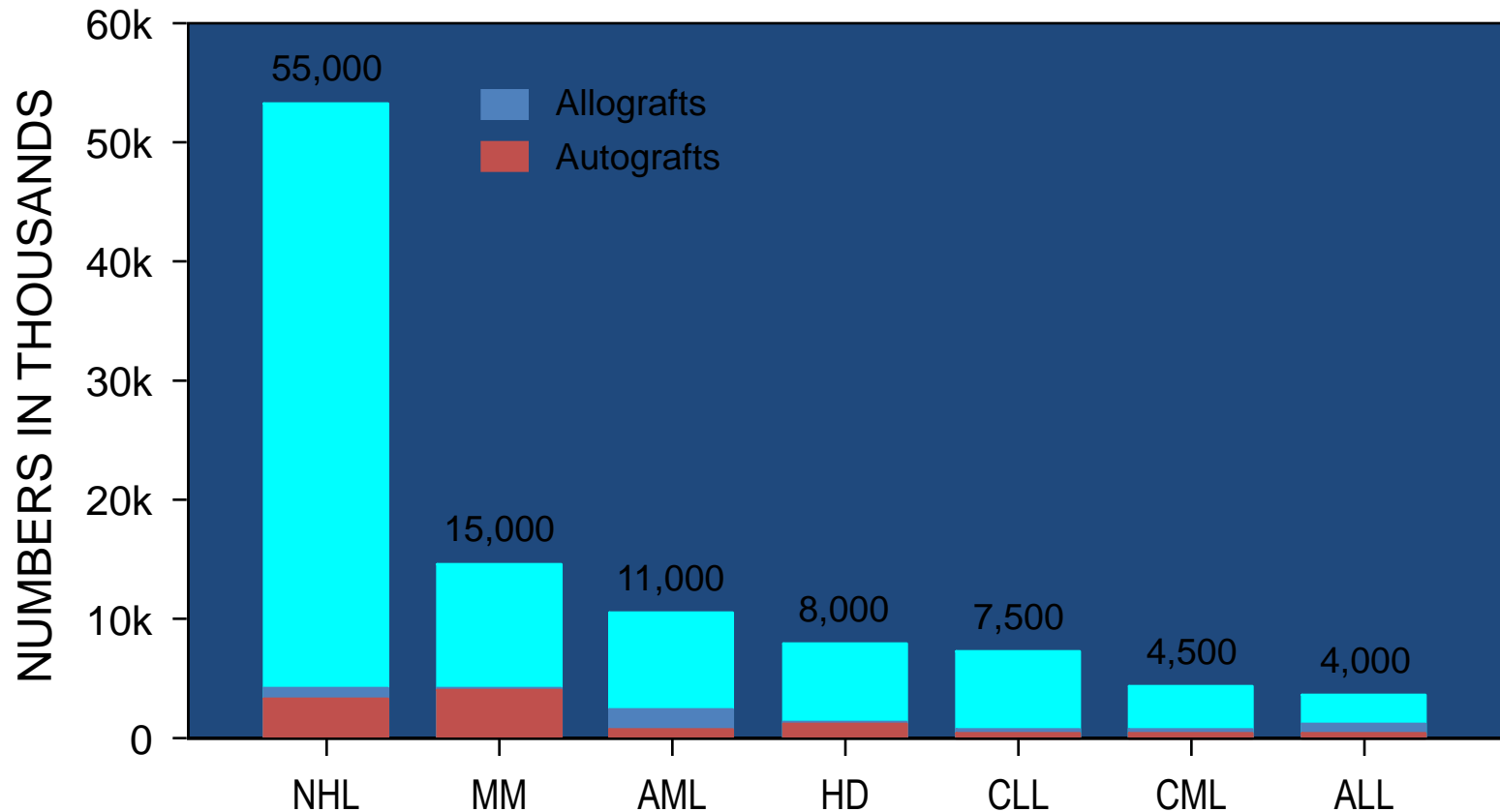
Indications for Allogeneic Hematopoietic Stem Cell Transplants (HCT), Registered with CIBMTR



Considerations for Use of HSCT

- High risk, high reward treatment
 - Substantial risk of treatment related morbidity from GVHD, and mortality (10-30%)
 - In many situations, HSCT provides the greatest chance of long term survival and cure
 - Indications for use and timing of HSCT constantly evolving with advances in chemotherapy and with HSCT
 - Precise indications often controversial and debated.

ESTIMATED NUMBERS OF POTENTIAL TRANSPLANT CANDIDATES vs TRANSPLANT RECIPIENTS IN U.S.



THE PROBLEM

Only about 15% of patients under the age of 70 years with hematologic malignancies considered treatable by HCT go on to have a transplant

Fewer than 10% of those with a disease (e.g. acute leukemia) for which **allo**transplant is considered the best HCT approach will have an allotransplant

Medical issues

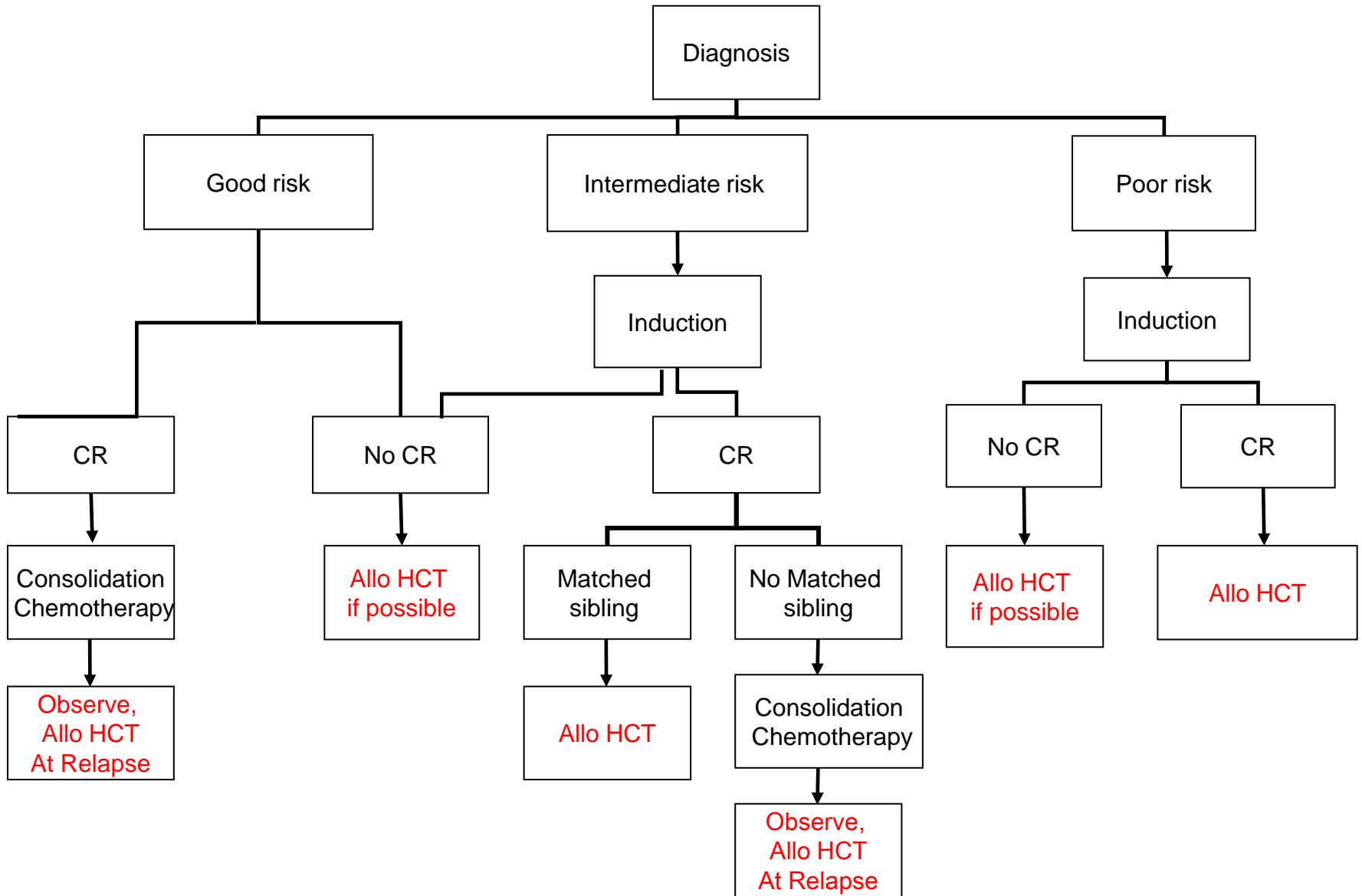
Age, disease status, HLA matched donor,
Comorbidities

Physician Referral

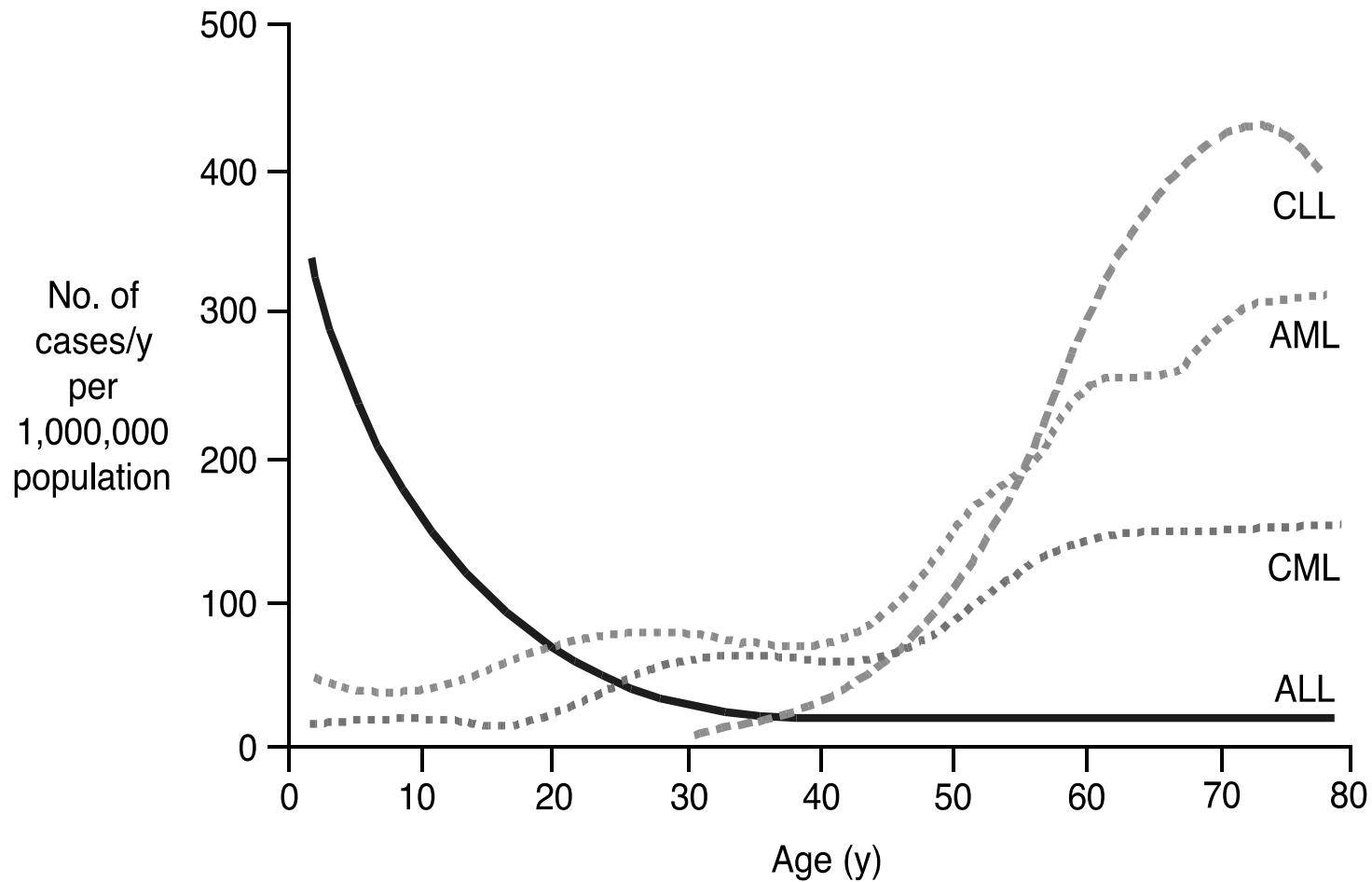
Socio-Economic Barriers

Travel/stay ~4 months at Transplant Center

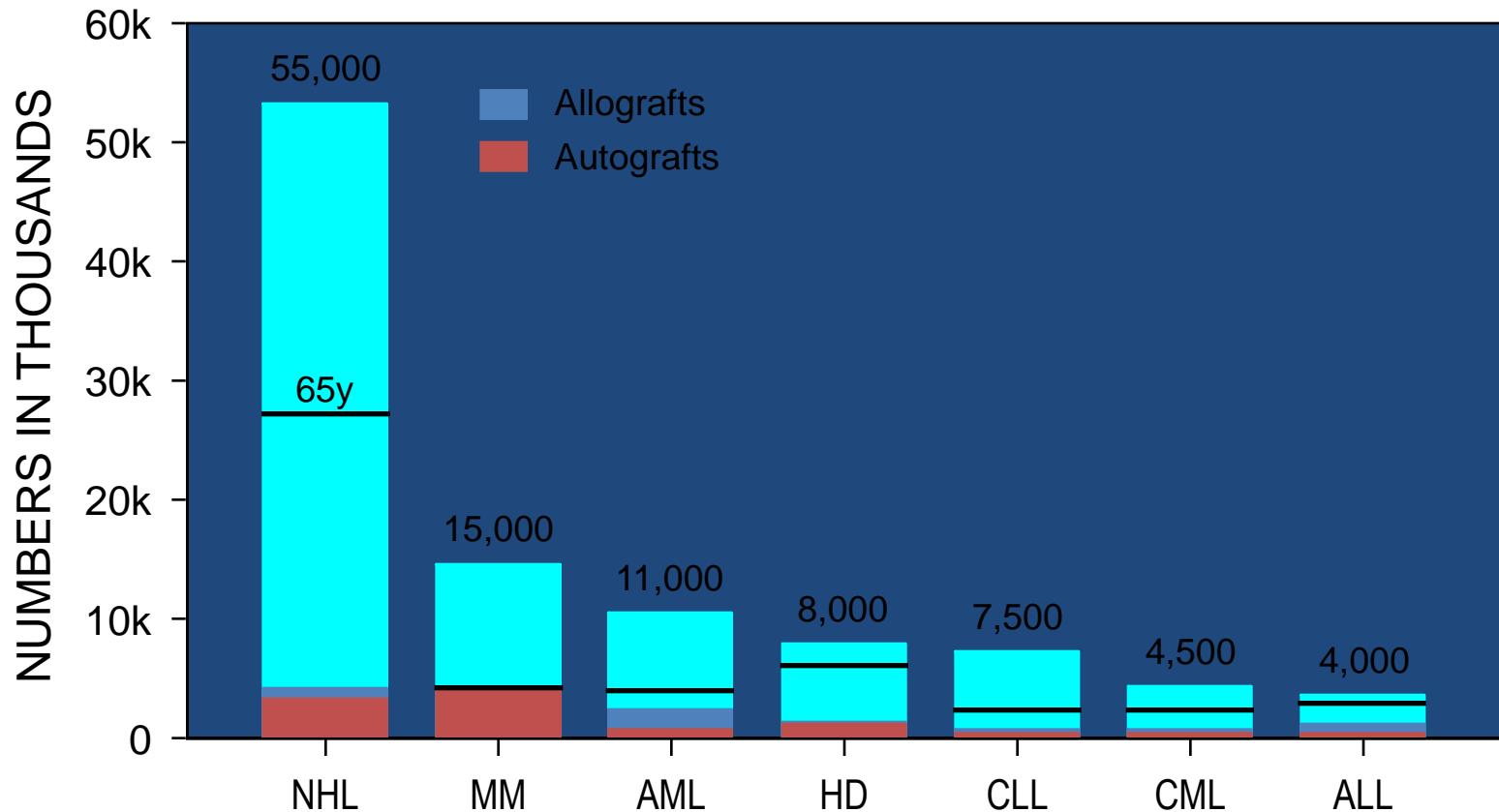
Newly Diagnosed AML Age <60



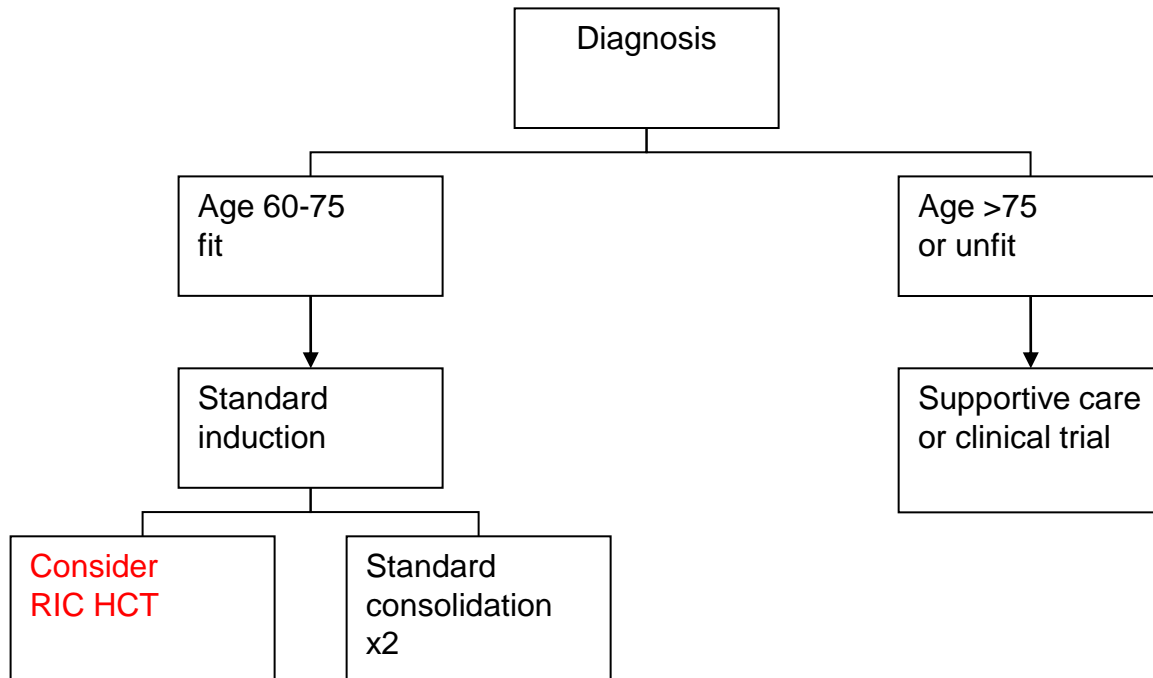
Incidence of Leukemias — By Age

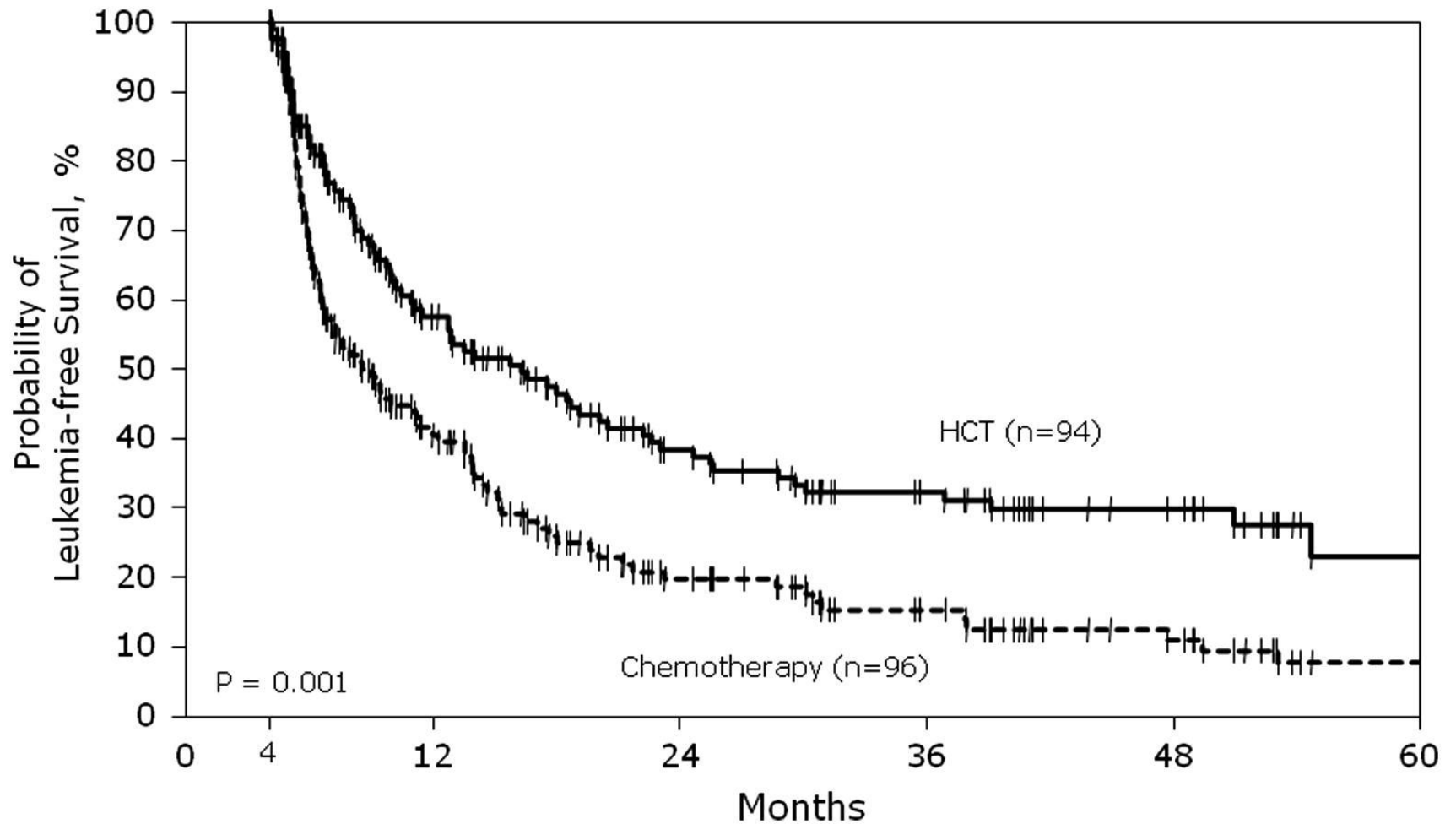


ESTIMATED NUMBERS OF POTENTIAL TRANSPLANT CANDIDATES vs TRANSPLANT RECIPIENTS IN U.S.



Newly Diagnosed AML Age >60





Medical Insurance Coverage- NMDP Forums

- Examined “Value” of HSCT and reimbursement
- Reviewed Medicare/medicaid coverage
- Describe optimal commercial insurance coverage description

Benefit Plan Recommendations

- Benefit plan should include access to a “centers of excellence” program for bone marrow/stem cell transplants,
- Key Medical Benefit Recommendations
 - rigorous qualification process for both adult and pediatric programs.
 - In addition to covering pre-transplant, transplant and posttransplant care as recommended by the transplant center, the benefit plan should cover donor search and typing costs, including:
 - – Full cost of biological sibling typing;
 - – Full cost of unrelated donor search, including typing and testing of potential donors, through the National Marrow Donor Program (NMDP) or other approved registry;
 - – Full cost of related donor procurement, including travel and lodging of the selected related donor for the donation process; and
 - – Full cost of donor cell product procurement for the unrelated donor.
 - Benefit plan should cover nutrition counseling and medical nutritional therapy for individuals with a diagnosis of cancer.

Benefit Plan Recommendations 2

- Key Medical Benefit Recommendations
- • Benefit plan should cover dental prevention services and treatments in the medical plan when such services are required prior to, during or after cancer treatment or SCT, and when not otherwise covered by the dental benefit.
- • Benefit plan should cover standard fertility preservation treatments when a medically necessary treatment may cause infertility.
- • Stop-loss insurance should apply benefits in a way that is consistent with the company's health care plan, including coverage of clinical trials and off-label use of drugs. Approved clinical trials should not be excluded under "experimental and investigational" language.
- Reasonable out-of-pocket thresholds should be established so that cost is not a significant barrier for patients to obtain their Key Pharmacy Benefit Recommendations
- medications. (Max of \$100 per script and aggregate \$200 per month)
- • Medical plans, pharmacy benefit plans and specialty pharmacy benefit plans should cover evidence-based cancer treatment, whether paid under the medical or pharmacy benefit. This includes coverage for off-label use of drugs and biologics when supported by evidence, as indicated in NCCN Guidelines.
- • Benefit plan should establish parity of patient cost-sharing between the medical and pharmacy benefits.
- Travel, housing costs

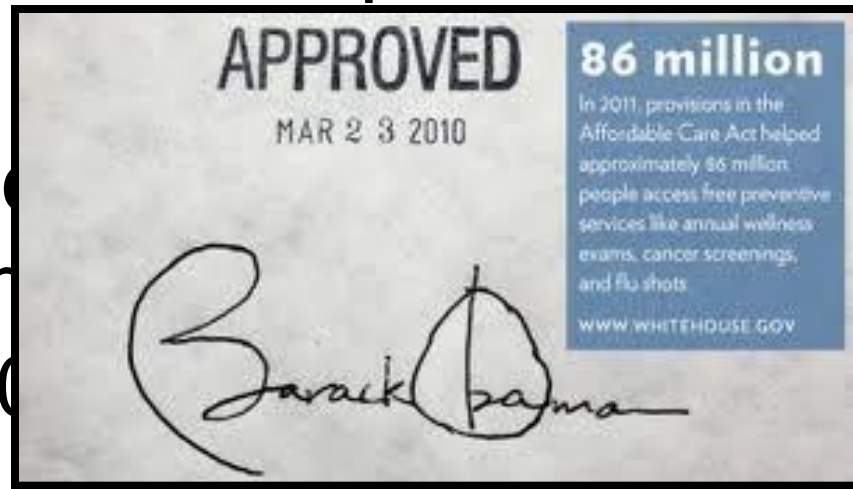
	Donor Search	Cell Procurement	Cell Infusion/HCT	Travel/Lodging	Length of Stay	Medications	Clinical Trials
Recommended Benefits	<p>Unrelated donor: coverage of tissue typing/testing through NMDP or other approved registry</p> <p>Related donor: coverage of tissue typing</p> <p>No limit on typing/testing costs if potential donors are in covered categories</p>	<p>Unrelated donor: full coverage of procurement; no limit.</p> <p>Related donor: full coverage of procurement, including travel and lodging of selected donor</p> <p>Autologous collection: full coverage of preparation, harvest and storage of cells</p>	<p>Coverage of HCT and subsequent therapeutic infusions for all medically necessary indications</p>	<p>Full coverage of travel and lodging costs for member and caregiver(s) for the transplant visit, in addition to necessary pre- and post- transplant evaluations</p> <p>Cover costs for second caregiver if patient is under 18 years of age</p>	<p>No limit on inpatient days or clinic visits</p>	<p>Coverage of all necessary medications throughout the HCT process, including post-transplant medications, without co-payment or co-insurance</p>	<p>Coverage of clinical trials appropriate to patient's stage, indication and clinical condition</p> <p>Minimum: coverage of routine care for patients on clinical trials, per the requirements in the <i>Affordable Care Act</i></p>
Rationale	<p>Amount of testing needed to find donor is based on genetics of patient; 70% of patients need an unrelated donor</p> <p>Limiting or excluding search coverage can negatively affect transplant outcomes and may result in unnecessary and costly complications</p>	<p>Obtaining cells for transplant is a necessary part of process</p> <p>For allogeneic HCT, cost dependent on donor location and type of cells selected for transplant</p>	<p>Transplant indications are expanding rapidly and improving lives of patients with otherwise fatal conditions</p> <p>Limiting access to transplant as treatment may result in increased costs and poor patient outcomes, including death</p>	<p>Patients may have to travel to center able to treat condition and/or within insurance network. Allogeneic HCT stay may be up to 100 days after cell infusion</p> <p>Limiting travel/lodging benefits may create difficulty in affording other necessary medical and/or living expenses</p>	<p>Several inpatient visits are often needed for treatment of primary disease, preparation for transplant and recovery; length of stays varies by disease, condition, graft success and complications</p> <p>Limiting inpatient days is counterproductive to treatment and may be life-threatening.</p>	<p>Access to medications is critical for success of HCT</p> <p>Prohibitive co-payments or co-insurance on medications may result in non-compliance, poor outcomes, graft failure and/or expensive hospital readmissions due to infection or complications</p>	<p>Results of clinical trials improve care for all patients</p> <p>Limiting access to clinical trials slows improvements in standards of care. Paying for identical care outside of a trial has identical cost without gaining future benefit from trial outcomes</p>
Administrative Guidance	<p>Place search and procurement into separate benefits to ensure funds are available for each phase of activity</p> <p>Do not require proof of donor insurance denial of HLA and other donor tests. Donors should never be billed for recipient costs, per Medicare guidance; this can significantly delay treatment process</p>	<p>Place search and procurement into separate benefits to ensure funds are available for each phase of activity</p>	<p>Include all transplant benefits under general medical benefit spending; do not implement a separate transplant-only benefit and spending limit</p>	<p>Use discounted housing options offered by transplant centers</p> <p>Adopt IRS reimbursement guidelines for taxable amounts or allow flexible spending of patient allocation. Patient reports to IRS; 1099 form. Consider reloadable debit cards</p>		<p>Understand off-label use of medications is common for cancer care</p> <p>Review medication list and test claims to assess coverage and co-payments/ insurance costs before patient is discharged from initial hospitalization</p>	

Visit marrow.org/payor for overview of donor search costs.

The recommendations in this guide were developed by a multi-disciplinary stakeholder group convened by the National Marrow Donor Program®. The group included transplant physicians, representatives from national health insurance companies and transplant networks, and administrators from hospitals with HCT programs.

Introduction to the ACA

- The Patient Protection and Affordable Care Act became law in March 2010. PPACA became the ACA.
- Designed with phased implementation for preparation
- Health insurance benefit provision on January 1, 2010



ect

- 18
- 3 Major Tenets:

Essential Health Benefit Set

- Requires coverage of several high-level care categories
- BMT and other transplant types not specifically defined
- Components of BMT are covered in the categories



Exchanges

- Offer affordable health insurance options with the goal of increasing the proportion of people with health insurance
- Question regarding access of patients to centers of excellence for HSCT, particularly for lower cost plans

Insurance Coverage

- Lack of coverage by Medicare, Medicaid for specific diagnoses in which allogeneic SCT is documented to be effective
 - Myelodysplastic Syndrome*
 - Lymphoma
 - Myeloproliferative diseases
- Coverage to apply to all sources of HSCT
 - BM, PBPC, Cord Blood
 - Related, unrelated,
 - Matched and mismatched

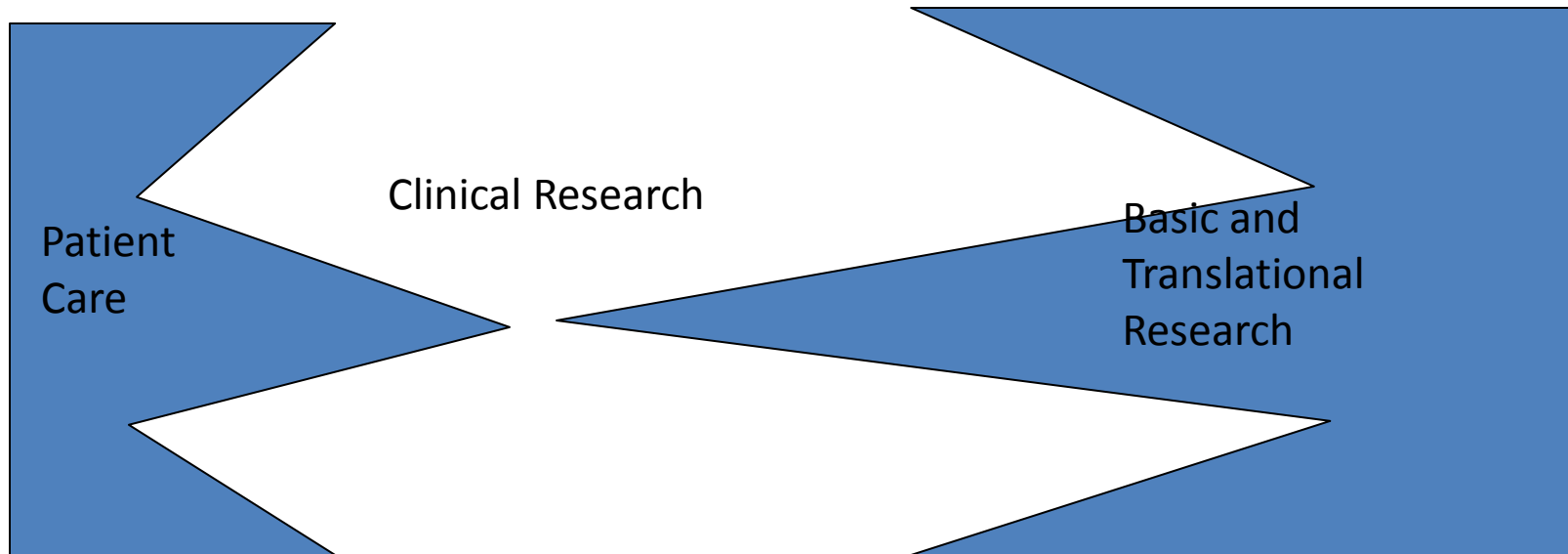
Recommendation

- The committee recommends that Medicare and Medicaid cover allogeneic hematopoietic transplantation for the following conditions
 - Lymphoma
 - Myeloproliferative diseases
 - Inborn errors of metabolism involving hematopoietic tissues
 - Hemoglobinopathies
- Allogeneic hematopoietic transplants should be approved for indications listed in NCCN guidelines or other generally accepted compendia

Recommendation

- Insurance plans should be required to cover the costs of HLA typing, donor search and donor transplant acquisition

Patient Care & Research



Research = Patient care

Clinical trials build upon standard of care to address major
Scientific questions, improve outcome, reduce morbidity and cost

Access to Clinical Trials

- Hematopoietic transplantation is a rapidly developing treatment. It remains a high risk treatment where clinical and scientific advances need to be rapidly translated to improve outcomes and standards of care
- Clinical trials provide access to advances and receipt of “cutting edge” in therapy; provides for quality improvement in medical treatment
- Clinical trials offer “best care”
 - Developed by experts, scientific and IRB review, monitored, early stopping criteria if does not meet expectations
- Many insurers do not cover participation in clinical trials
 - Insist on “standard of care treatment” often known to be relatively ineffective
 - Rationale uncertain. No evidence of increased cost, toxicity
- Coverage for Clinical Trials included as provision of ACA, but rules for enforcement not established.
- Medical care will never improve without clinical research

Affordable Care Act

- ACA mandates Clinical Trial Coverage for all plans as
- they renew January 1, 2014 and thereafter
- Requires coverage for Routine patient Costs
- Phase I, II, III or IV meet the definition
- Treatment of Cancer or life-threatening Disease
- Clinical Trial setting would not invalidate Routine Costs

Physician Issues

- Referring Physicians
 - Rapidly evolving indications for HSCT
 - Education regarding proper referral
 - Misperceptions re: Transplant toxicities and outcomes
 - Controversies in indications
 - Financial disincentives
 - Late referrals
 - Encourage Pay for Performance- doing the “right thing”.
 - United States is in the “middle of the pack” of industrialized countries in number of transplants per capita.
- Facilities/Personpower
 - Hospital/physician/staff insufficient for envisioned needs
 - Additional resources required for cord blood transplants
 - Search
 - Supportive care, particularly for adults
 - Requires rationing resources at transplant center

Patient/Family Issues

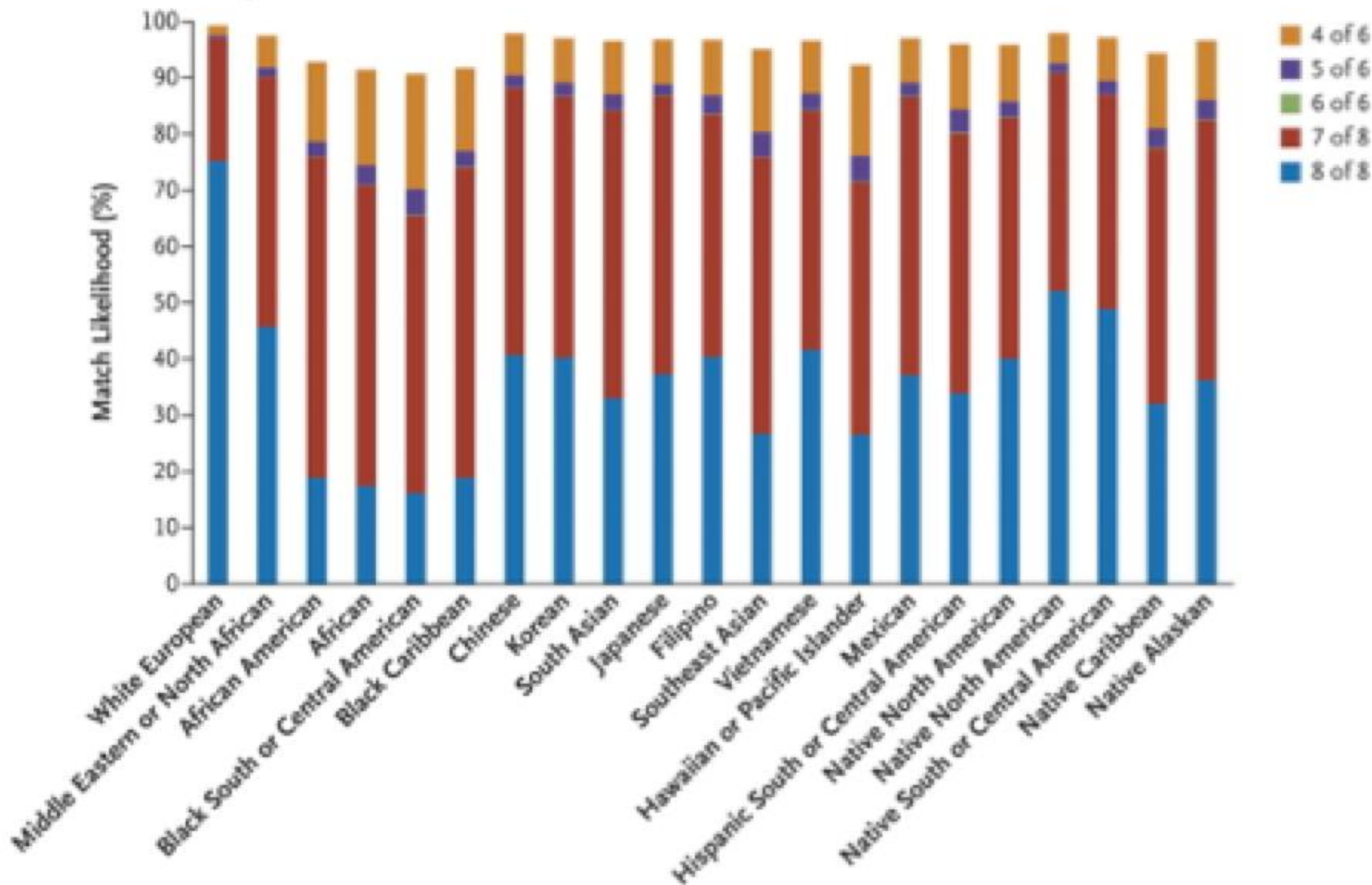
- Education/misperceptions
- Logistical Geographic-
 - no local transplant center, particularly rural patients
- Must go to transplant center for several months, unreimbursed expenses for travel, housing, loss of income
- Need for caregiver, emotional and financial support for caregivers

- Transplants are life changing events for the whole family.
 - Leave home
 - Loss of employment, income
 - Redirecting efforts as caregivers

Donor Issues

- Best results with HLA matched sibling donor
- Matched unrelated donor
 - Available for about half of patients, chances depend on ethnic origin
- Improved results with cord blood transplants and haploidentical transplants, so everyone has a donor.

B Patients ≥20 Yr of Age



ConsumerReportsHealth

MHQP
MASSACHUSETTS
HEALTH QUALITY PARTNERSHIP

Special Report for Massachusetts residents

How Does Your Doctor Compare?

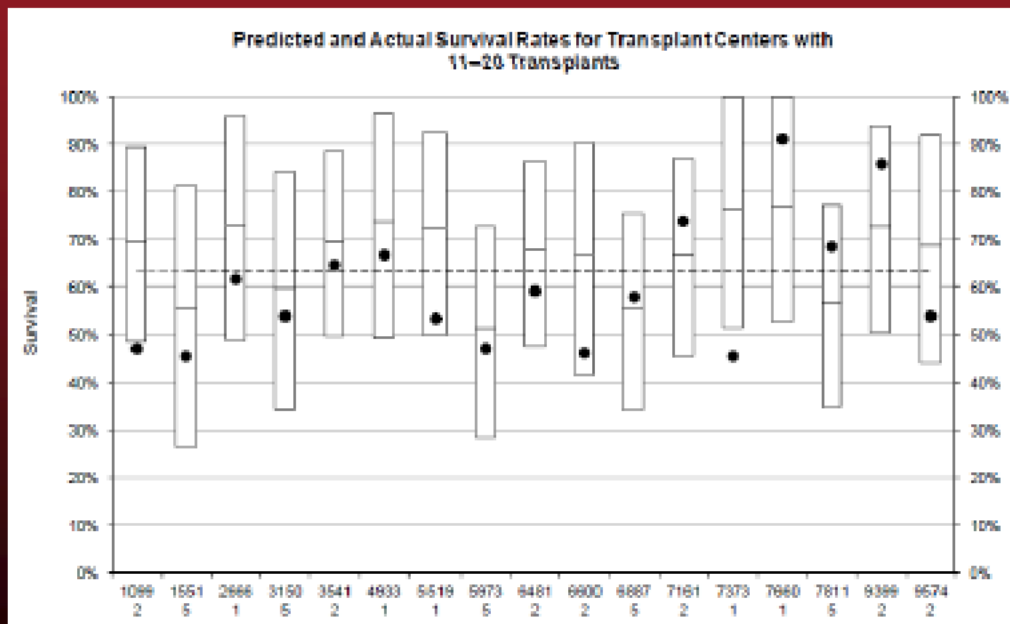
- ▣ **Exclusive:** Patients rate 487 adult, family & pediatric practices
- ▣ **How to get the best care**
- ▣ **Quiz: Does your physician measure up?**



**GUIDE TO
PRIMARY CARE
PHYSICIANS IN
MASSACHUSETTS**
PAGE 10

Center Specific Results

Reporting Results



Affordable Care Act

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Conclusion

- HSCT offers uniquely effective, potentially life saving treatment for a broad range of malignant and nonmalignant diseases
- Access of patients to this is essential. This is a particular concern for the economically disadvantaged and minority ethnic groups
- Continued dialog is necessary between professionals and organizations involved with HSCT with medical insurers and government payors to assure access and appropriate coverage.
- Ongoing professional and public education is needed regarding optimal use of HSCT