

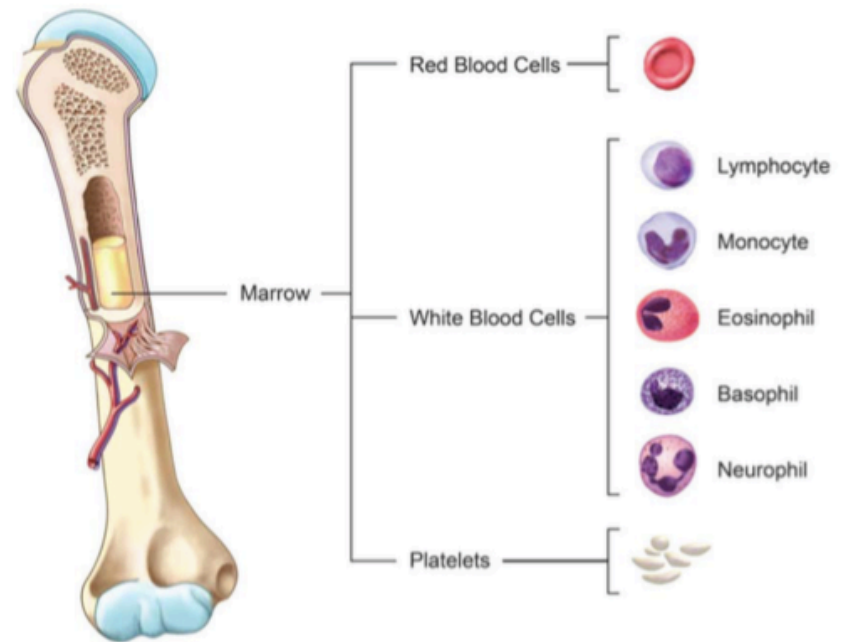
# Bone Marrow Transplant for MDS Patients

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- BMT: The basics
- BMT for MDS: The evidence
- Innovative approaches to BMT for MDS

# What is Bone Marrow?

- Marrow is the soft tissue inside bones that produces blood forming cells that mature into red blood cells, white cells and platelets (factory)
- **Red Blood cells** – carry oxygen through our body
- **White Blood cells** – help fight infection
- **Platelets** – help control bleeding

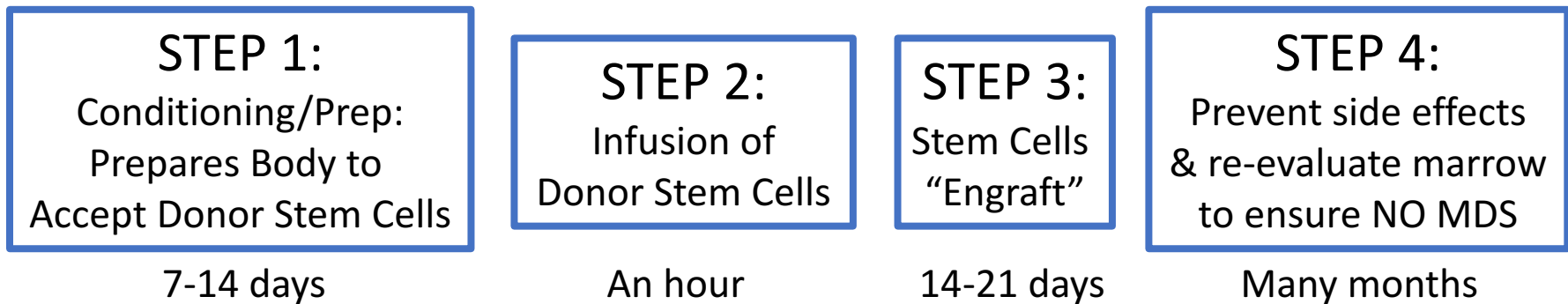


# What is an Allogeneic BMT?

- BMT= bone marrow (or stem cell) transplant
- Allogeneic= from a Donor
  - Someone “immunologically” compatible
- Transplant= Replace recipient (patient’s) bone marrow with the donor’s bone marrow
  - Replace RBC, platelets, WBC

# How Do We Perform Allogeneic BMT?

- Medical transplant= NO SURGERY



- How do we collect donor stem cells?
  - Bone marrow harvest in OR
  - Use stem cell booster and collect via peripheral veins

# Why Do We Perform Allogeneic BMT?

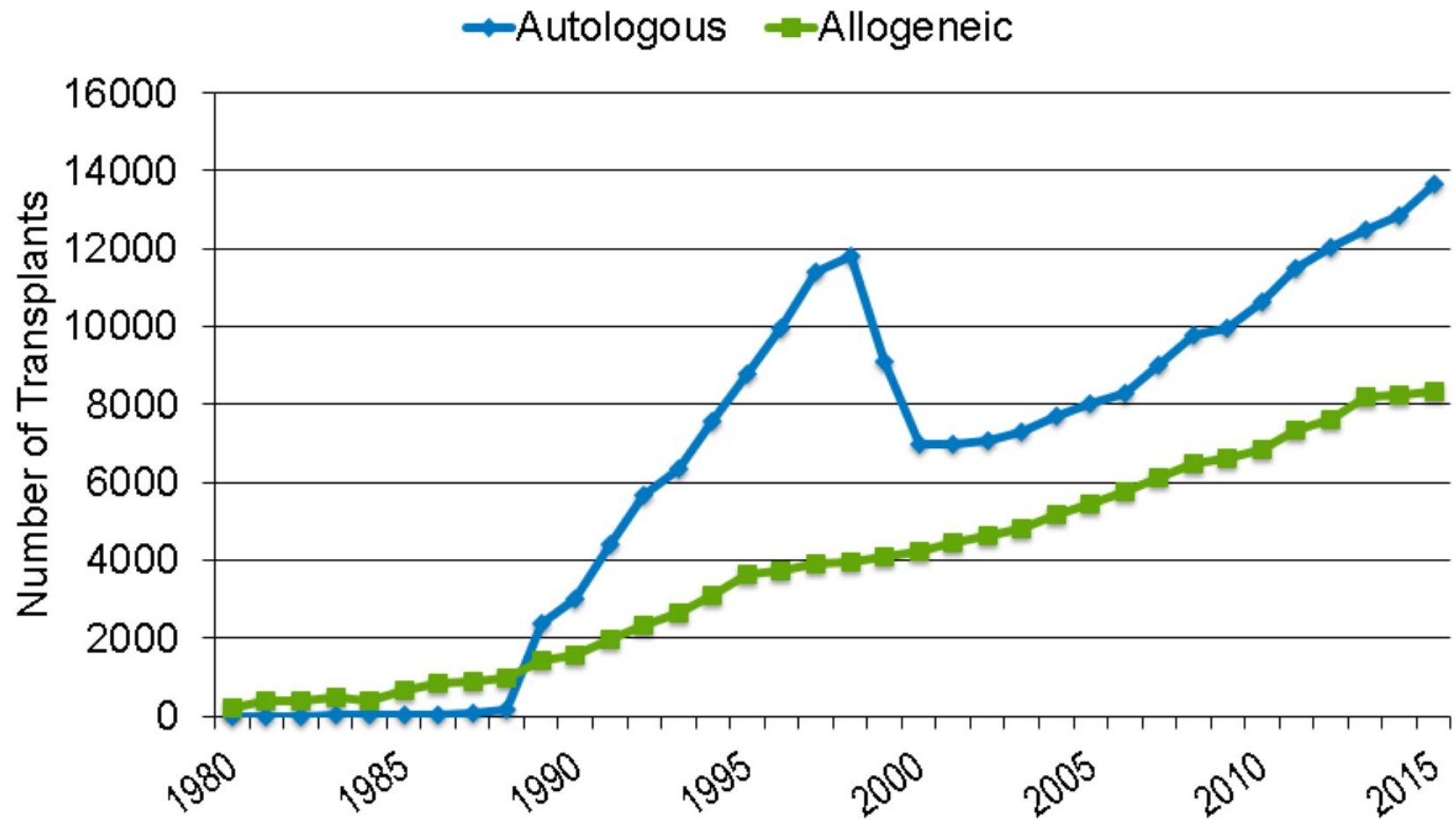
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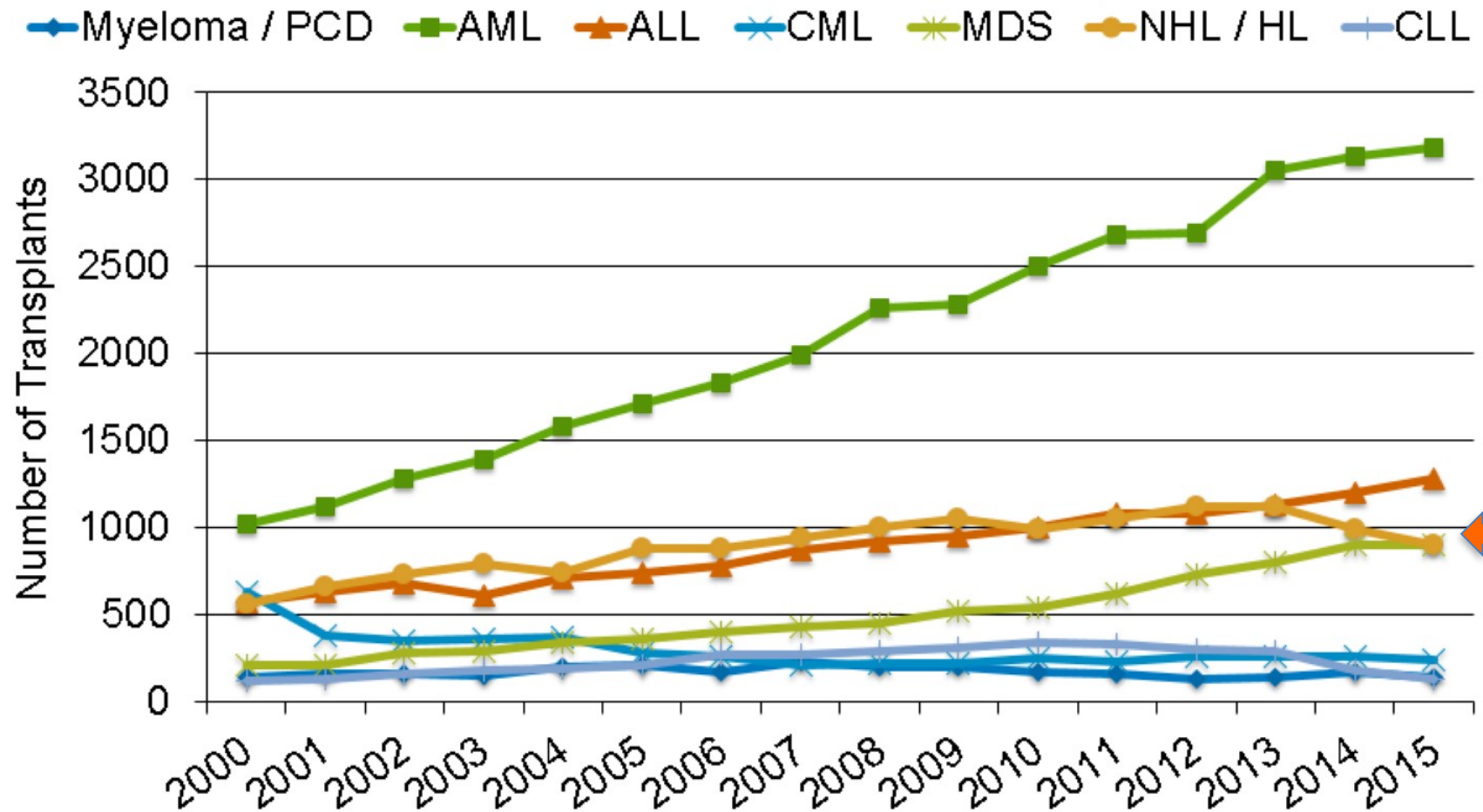
New Immune System

- New (donor) immune system can be very powerful at controlling blood cancer cells → CURE MDS

# Annual Number of HCT Recipients in the US by Transplant Type

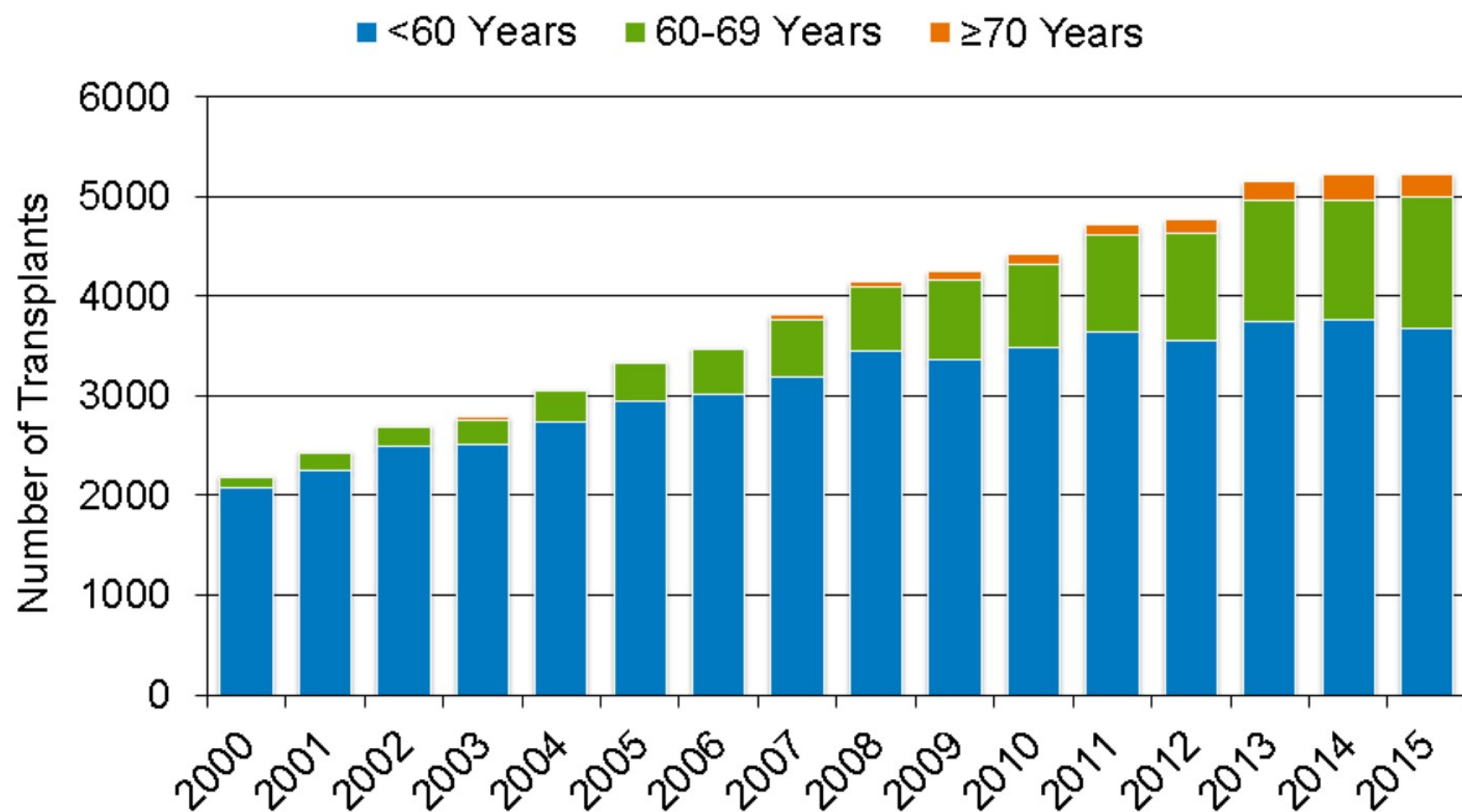


# Selected Disease Trends for Allogeneic HCT in the US



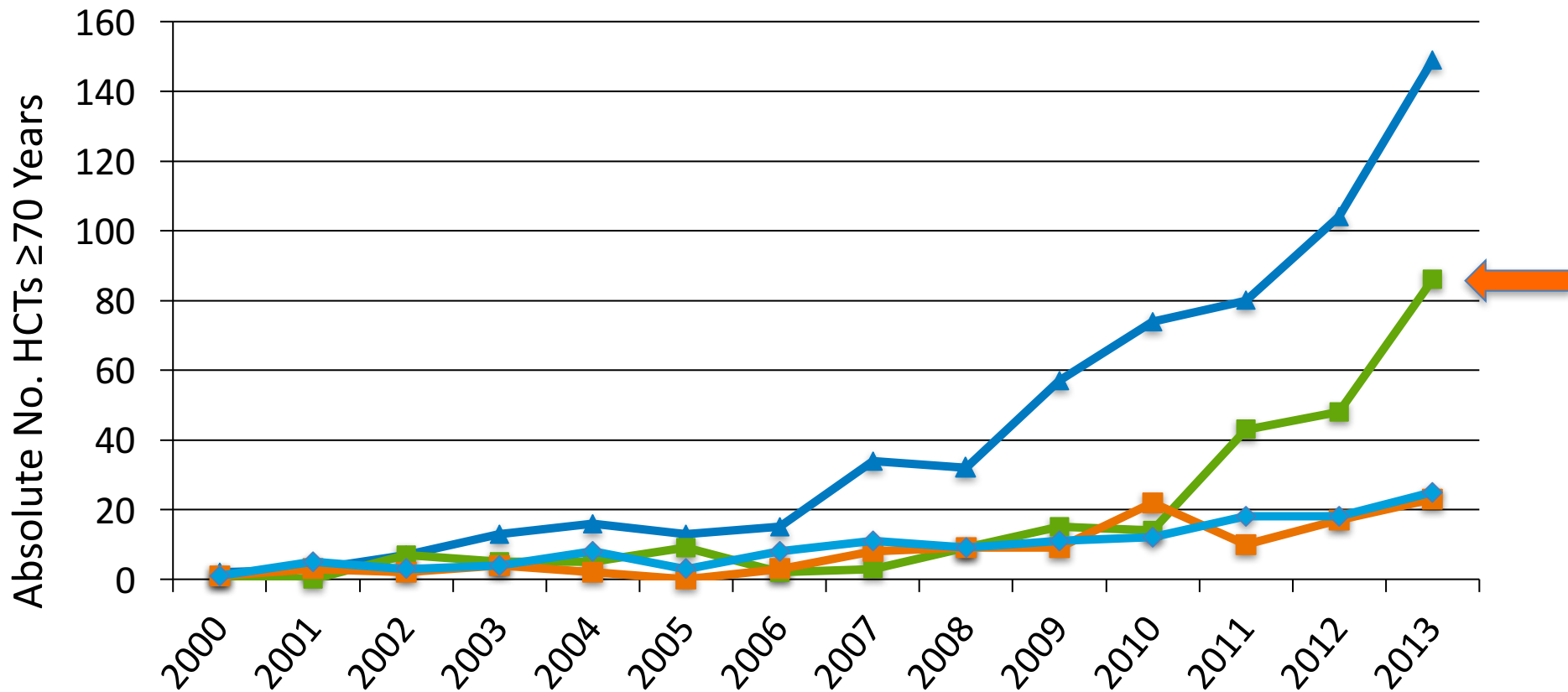


# Trends in Allogeneic HCT by Recipient Age<sup>^</sup>



# Trends in Allogeneic BMT Utilization for Adults $\geq 70$ Years, by Disease

AML MDS/MPS Non-Hodgkin lymphoma Others



# Why is Allogeneic BMT for MDS on the Rise?

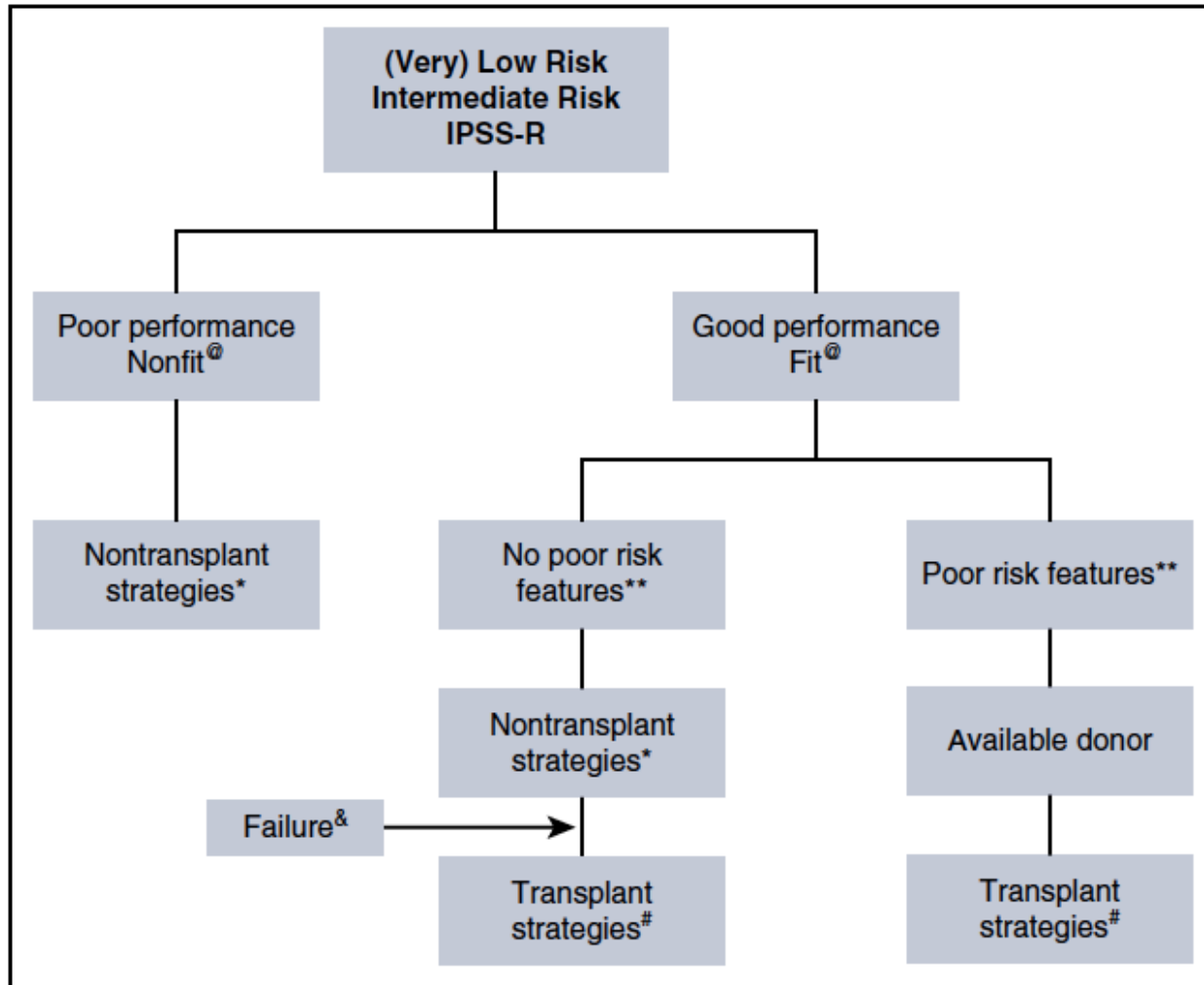
- Historically, patients 65 and older with Medicare did not have coverage for BMT for MDS.
- On August 4th 2010, the Centers for Medicare and Medicaid services (CMS) established coverage for BMT for MDS through coverage with evidence development (CED).
- A Center for International Bone Marrow Transplant Research (CIBMTR) study comparing outcomes of patients 55-64 vs. 65 and older was approved in December 2010.

# Medicare Coverage with Evidence Development

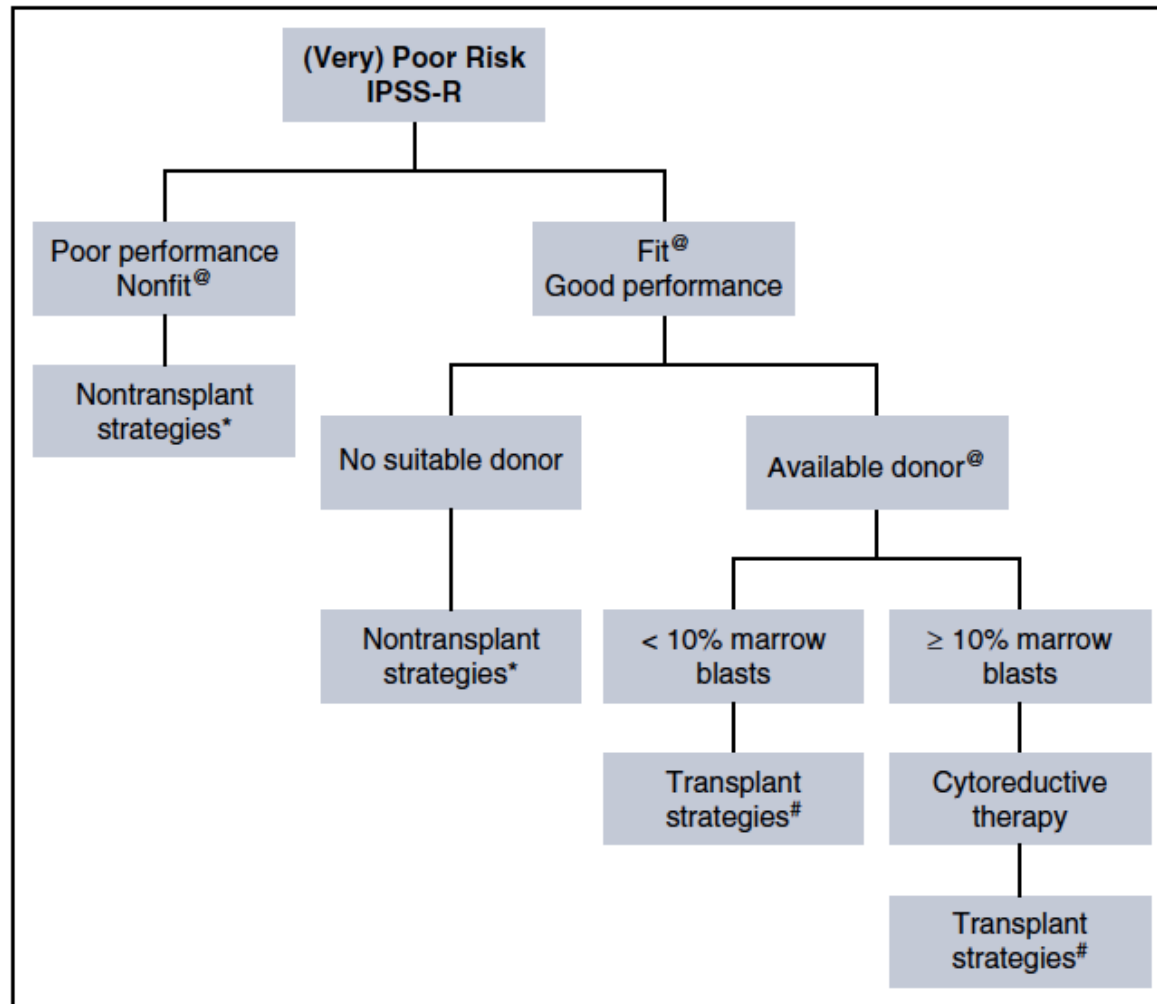
## MDS BMT Study

- The study compared the outcomes of:
  - 688 patients aged 65 and older and
  - 592 patients aged 55-64
  - who underwent allogeneic BMT for MDS from 2010-2014
- Survival at 100 days and at two years following BMT for MDS patients aged 65 and older is comparable to patients aged 55 to 64.
- Age alone should not be a determinant when considering BMT for patients with MDS.

# How Do We Determine Which MDS Patient to Transplant?



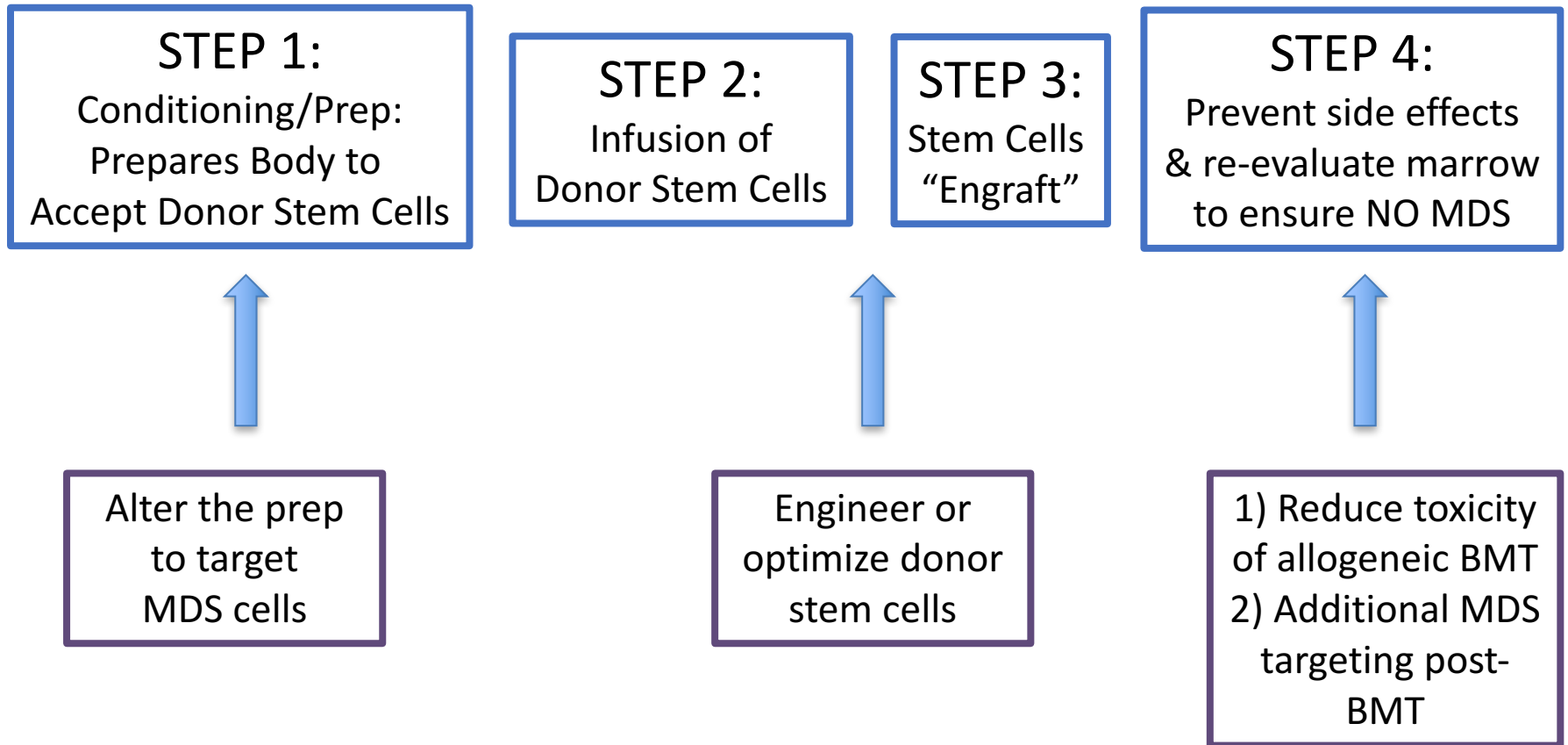
# How Do We Determine Which MDS Patient to Transplant?



# What Are Outcomes after BMT for MDS?

- In general....
  - 30-40% of intermediate/high risk MDS patients will be cured following allogeneic BMT
  - BUT
  - Some patients will have serious morbidity/mortality from the transplant process and some patients will have recurrence of progression of MDS after BMT

# How Can We Improve Allogeneic BMT for MDS?







Phase I/II Clinical Trial of an  
MDS Stem Cell Targeting Antibody  
Plus Low Intensity Conditioning for  
Patients with MDS  
undergoing Allogeneic BMT

# Reducing BMT Complications



## PROGRESS trial

Prevention and Reduction Of Gvhd and  
Relapse and Enhancing Survival after  
Stem cell transplantation



- Phase III clinical trial conducted across the US aiming to improve post-BMT outcomes for patients with MDS and acute leukemia by reducing transplant toxicity.

# Conclusions

- Allogeneic BMT is an immunotherapy that offers a potential for cure for intermediate/high risk MDS patients
- The use of allogeneic BMT for MDS (and for older adults) is rising
- New and innovative approaches to BMT are needed to further improve outcomes