COUNCIL MEETING
Sharing Our Passion for Life

BMT CTN History and Scientific Impact

Council Meeting 2016

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Disclosures

The following faculty and planning committee staff have no financial disclosures:

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Ashley Spahn	CIBMTR
Stephen Spellman	CIBMTR
Del Steckler	NMDP – Be The Match

Learning objectives

At the conclusion of this session, attendees will be able to:

- Describe the development of the BMT CTN.
- State the impact of the BMT CTN on the hematopoietic stem cell transplant community.
- Summarize important finds from BMT CTN clinical trials.
- Describe BMT CTN usage of patient reported outcomes in clinical trials.

BMT CTN: A Model of a Focused (and Successful) Clinical Trials Network

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

BMT Clinical Trials Research in the United States in the 1990s

- Largely single institution
  - Investigator initiated
  - Mostly Phase I & II exploring new strategies
  - R01 or P01 funded
  - Few Pharma-funded
- Few multi-center trials
- Few definitive trials

- Challenges in BMT
  - Small, heterogeneous population
  - Multiple competing risks thus unattractive setting for pharma to test new drugs
  - NCI funded Cooperative Groups focused on cancer; not transplantation

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

Blood and Marrow Transplant Clinical Trials Network (BMT CTN)

- Established: Sept. 2001; renewed 2006, 2011
  - 20 Core Centers/Consortia
  - >80 Affiliate Centers
  - 1 Data and Coordinating Center
- Goal of the Program:
  - Provide the infrastructure needed to allow promising HCT therapies to be developed/evaluated in high quality multicenter studies

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Advantages of a Network

- Greater opportunity to enroll patients
  - More likely to reach target enrollment
  - Address problems requiring larger trials
- Greater opportunity for patients to have access to trials
- Uses heterogeneity of the community to gain validity – new approaches tested in the broad range of transplant centers where it will be applied
- Shared ideas and resources for research

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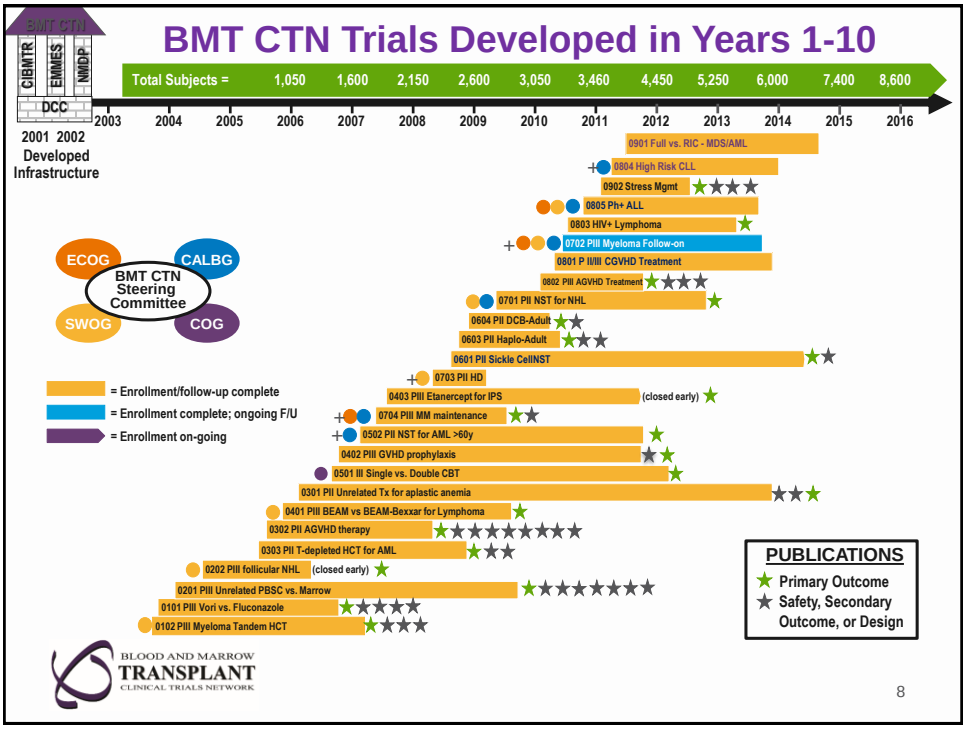

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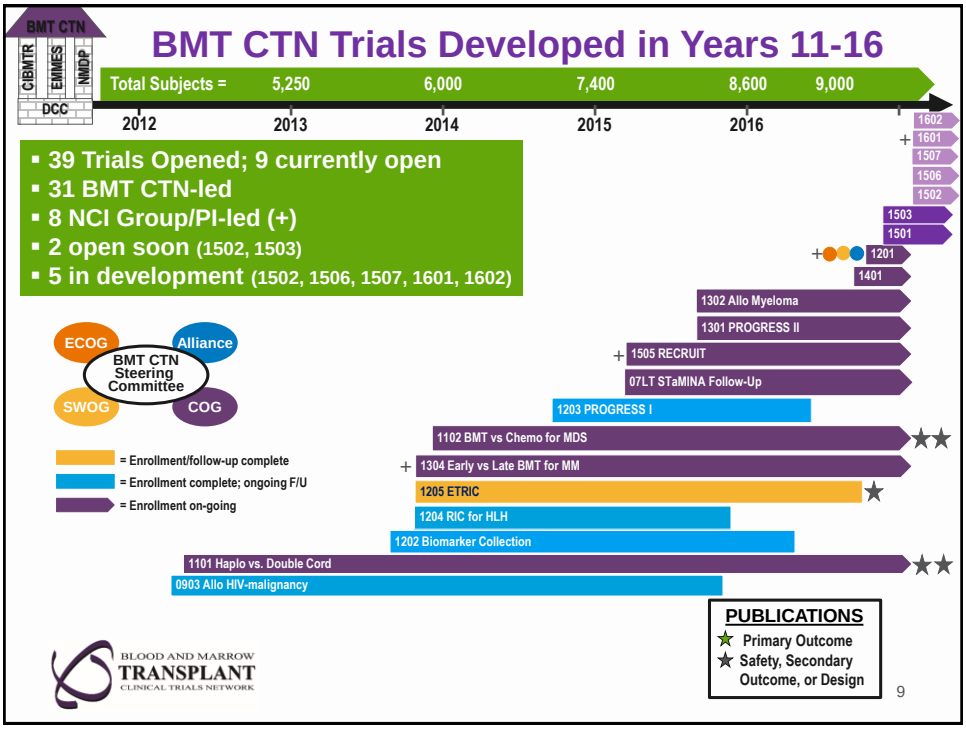

Challenges of a Network

- Requires investigator cooperation—
  Their ideas may not be adopted
- Requires individual center cooperation
  Their trials may have to wait
- Requires
  - Infrastructure for data collection, auditing and analysis
  - Monitoring outcomes for safety
  - Flexibility and adaptability to new ideas

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Elements of BMT CTN's Success

- Streamlined infrastructure
  - Shared decision making
  - DCC with integrated medical expertise
  - Continuous efforts to improve
- CIBMTR Database
- Inclusivity/collaboration
- Financial support/stewardship

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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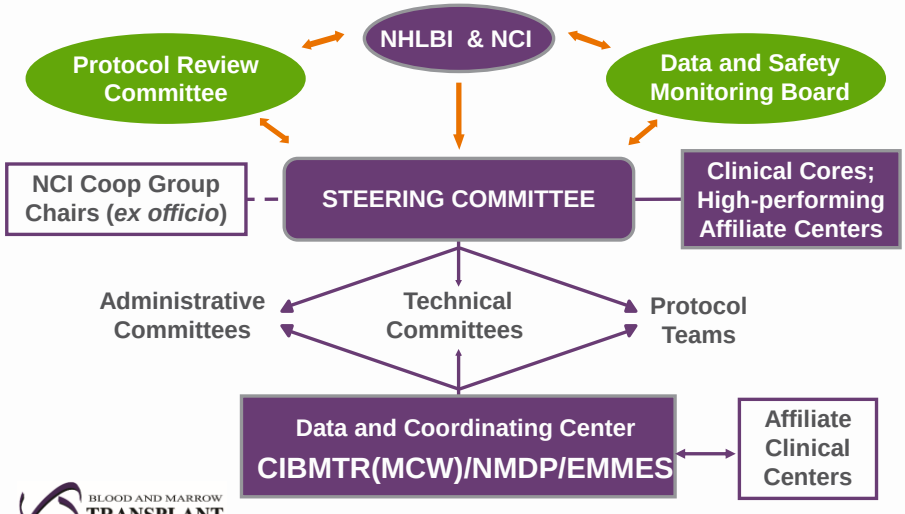
BMT CTN Organizational Structure



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Rotating Leadership positions

- Vice-chair elected every two years
  - Serves 2 years as vice-chair, 1 year as chair-elect, 2 years as chair, 1 year as immediate-past chair
  - Provides 3 people to interact with DCC (and NIH) on a regular basis
  - Gives a lot of people experience with the challenges of running the Network

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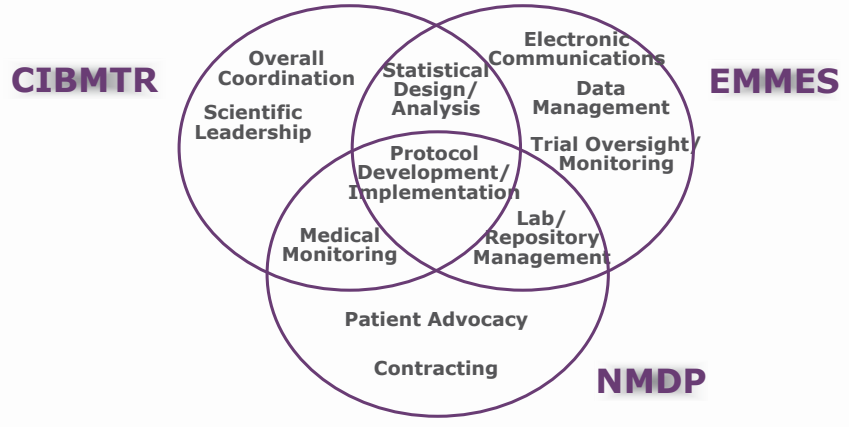
RESPONSIBILITIES AND INTERACTIONS OF DCC MEMBERS



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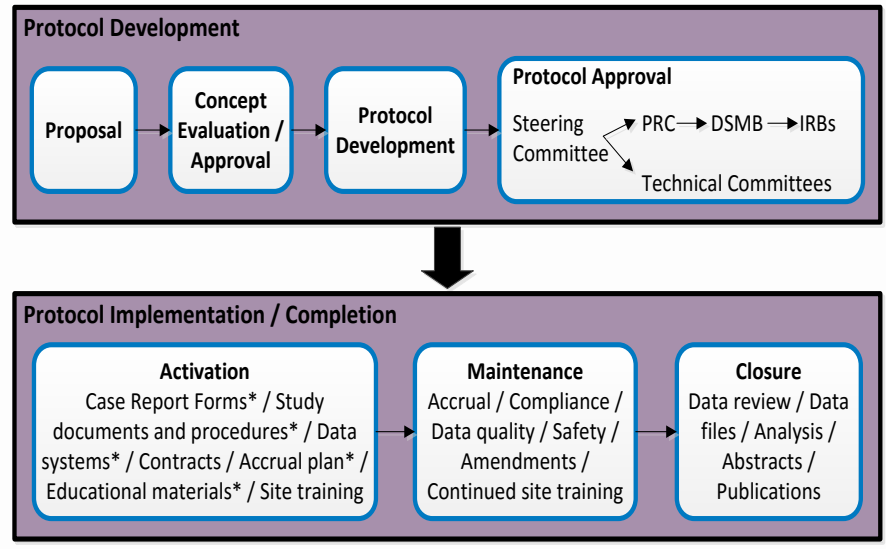

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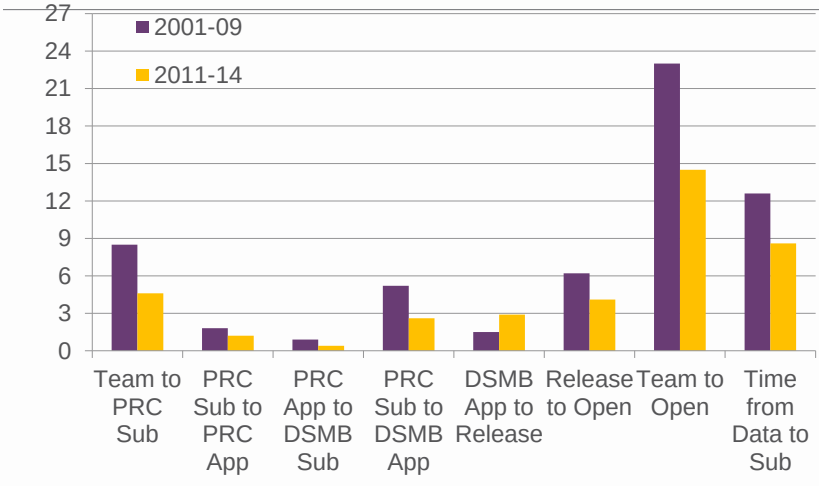
Network Activation Timeframes



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Elements of BMT CTN's Success

- Streamlined infrastructure
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- CIBMTR Database
- Inclusivity/collaboration

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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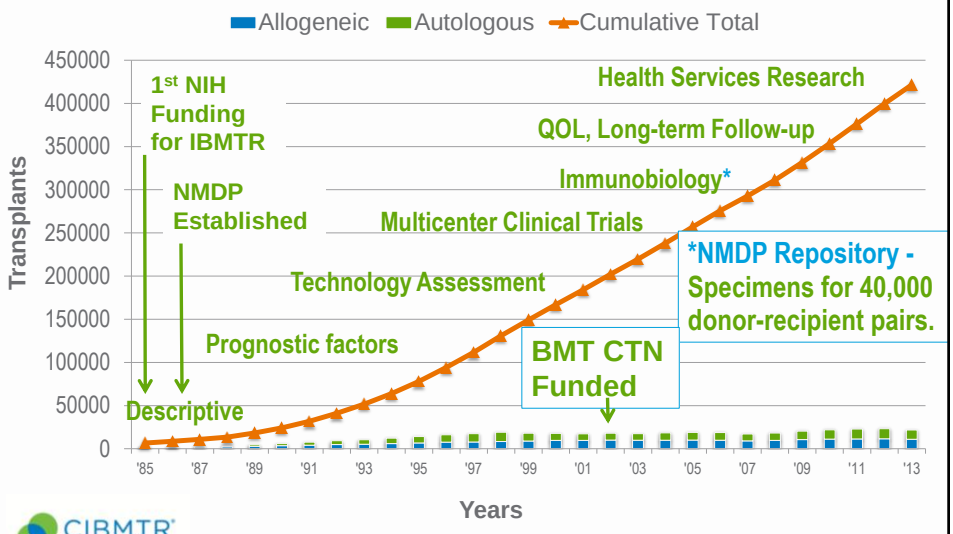
CIBMTR: 440,000 Cases Registered, up to ~10,000 variables per person (most with repeated observations, some extending over >30 years), >1000 publications



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BMT CTN Specimen Inventory

BMT CTN Protocol	# Aliquots Stored
0701	95
0702	41,454
0801	4,654
0802	3,645
0901	1,391
1101	9,802
1102	2,908
1202	259,382
1203	6,844
1204	3,252
1301	58
1302	150
TOTAL	333,635

CIBMTR
CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

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Elements of BMT CTN's Success

- Streamlined infrastructure
  - Shared decision making
  - DCC with integrated medical expertise
  - Continuous efforts to improve
- CIBMTR Database
- Inclusivity/collaboration

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CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

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Core Centers	
Baylor, Houston Children's National, Washington, DC	City of Hope, Duarte
Case Western, Cleveland Oregon Health Sciences (Adults), Portland Cleveland Clinic West Virginia University, Morgantown	Dana Farber, Boston Brigham & Women's, Boston Mass General, Boston Boston Children's
Duke, Durham, North Carolina	Fred Hutchinson CC, Seattle
Moffitt CC, Tampa	Johns Hopkins, Baltimore
Memorial Sloan-Kettering CC, New York	Northside Hospital, Atlanta
Ohio State, Columbus Roswell Park CC, Buffalo Univ North Carolina, Chapel Hill Univ California-San Francisco Virginia Commonwealth,, Richmond	University of Michigan, Ann Arbor Mayo Clinic, Rochester, Minnesota Mt. Sinai Hospital, New York
Pediatric Blood & Marrow Transplant Consortium, 70 centers in the US and Canada	Univ Florida, Gainesville Emory University, Atlanta, Georgia
Stanford Hospital, Palo Alto	MD Anderson, Houston
Univ Minnesota, Minneapolis	Univ Pennsylvania, Philadelphia
Univ Nebraska, Omaha Univ Kansas, Kansas City	Washington Univ, St. Louis

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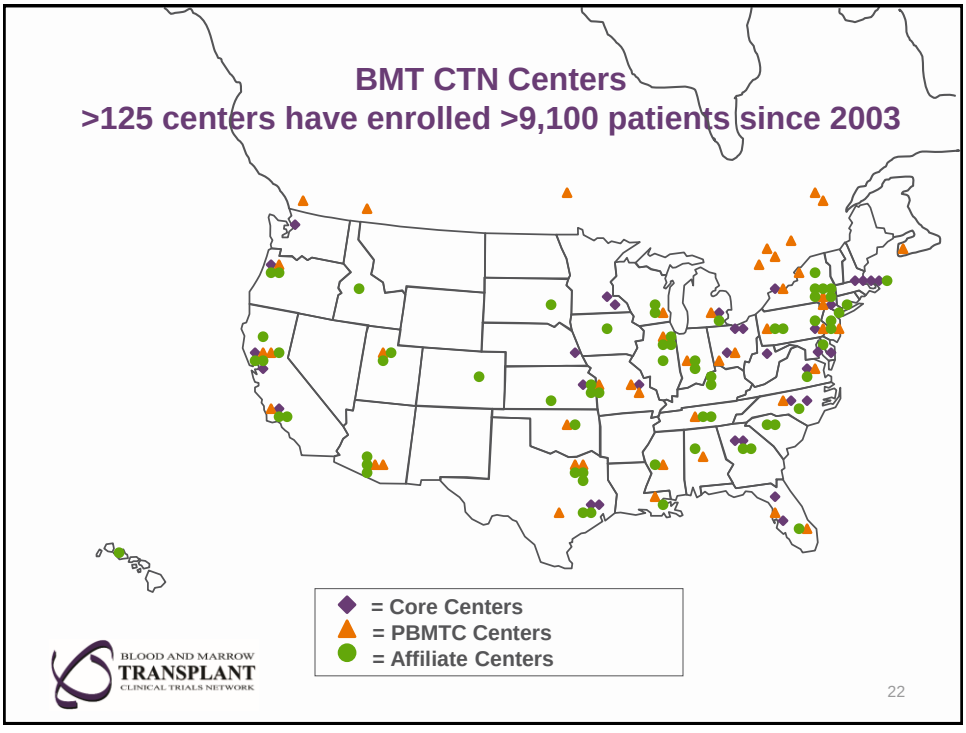
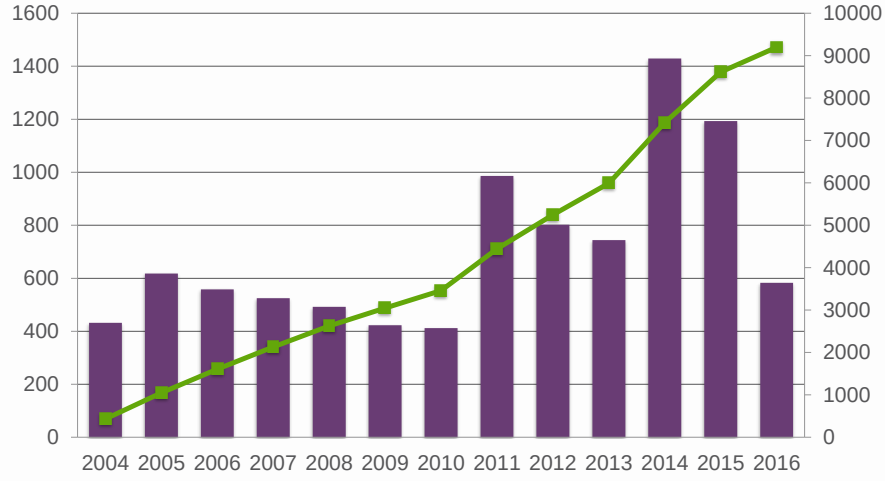


BMT CTN Yearly and Cumulative Accrual to all Protocols, 2004-2016 >9,100 patients



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Protocols with Major Public-Private Partnerships

- 0303 – Miltenyi: pivotal phase 2 trial that led to preliminary approval of CD34 selection device (assisted by comparison to a non-T-cell depleted cohort from CIBMTR)
- 0702 – Celgene, Millenium: posttransplant consolidation for myeloma
- 1301 – Miltenyi: phase 3 registration trial of CD34 selection device
- 1506 – Astellas: registration trial of gilteritinib for maintenance after allotransplant for flt3+ AML
- 1602 – Gilead: filgotinib for treatment of high risk acute GVHD

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BMT CTN TRIALS - SUMMARY

	All Trials	Phase II	Phase III
Donor/Graft Source	13	8	6
GVHD	7	5	4
Infection	3	2	2
Disease Control	15	12	8
Regimen Toxicity	5	8	4
QOL	8	3	4
TOTAL	39*	18	18

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

Network Productivity

	Funding Periods			
	2001-5[a]	2006-10	2011-16	Totals
Trials Opened	7	16	16	**39**
Primary results papers	0	3	16	19
Ancillary papers	0	3	24	27
Methodologic papers	2	1	3	6
Other publications	1	6	9	16
Total Publications	3	13	52	**68**

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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BMT CTN

- Major findings
- Highlights of important trials
- Impact and future influences on the field

Daniel Weisdorf MD
University of Minnesota

Transplant Questions Addressed

- Best prevention and treatment for GVHD
- Best treatment strategy
- What type of transplant or graft or conditioning regimen for specific diseases

- Best supportive care/quality of life

BMT CTN Major trials

- GVHD Treatment: more drugs vs. fewer
  - GVHD prophylaxis: drugs or graft manipulations
- Conditioning intensity
  - Radioimmunotherapy added for NHL autografts
  - Myeloablative vs. Reduced Intensity Conditioning
- Infection prevention: Fluconazole vs. Vori
- Myeloma: several approaches
- Graft choices
  - Haplo vs. UCB Reduced intensity transplants
  - Single vs. Double UCB for Children
  - BM vs. PBSC for URD transplants

GVHD Treatment: BMT CTN 0302 & 0802

Initial systemic treatment of acute GVHD: a Phase II randomized trial evaluating

etanercept, mycophenolate mofetil, denileukin diftitox (Ontak), and pentostatin

- Previously– nothing was better than steroids alone for treating new acute GVHD

[***wished we could change practice***].

aGVHD Response at Day 28

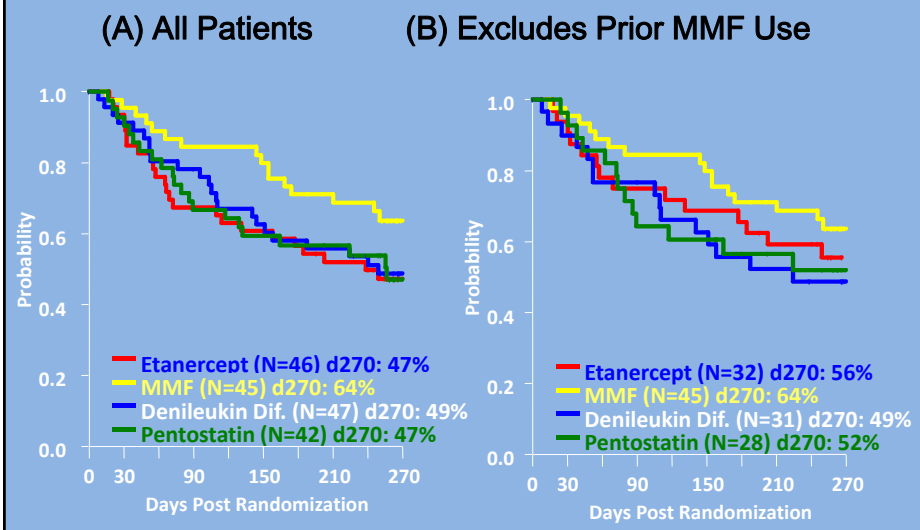
	Etanercept N=46	MMF N=45	Denil N=47	Pentostatin N=42
CR (Overall)	26%	60%	53%	38%
Skin	33% (12 / 36)	60% (21 / 35)	49% (17 / 35)	41% (14 / 34)
Lower GI	33% (4 / 12)	67% (12 / 18)	36% (5 / 14)	41% (7 / 17)
Upper GI	50% (5 / 10)	92% (11 / 12)	71% (10 / 14)	62% (8 / 13)
Liver	33% (2 / 6)	71% (5 / 7)	43% (3 / 7)	40% (2 / 5)
CR (excl. prior MMF)	28%	60%	48%	39%
CR or PR	48%	78%	60%	62%
Progression	15%	2%	6%	10%

Response at Day 56

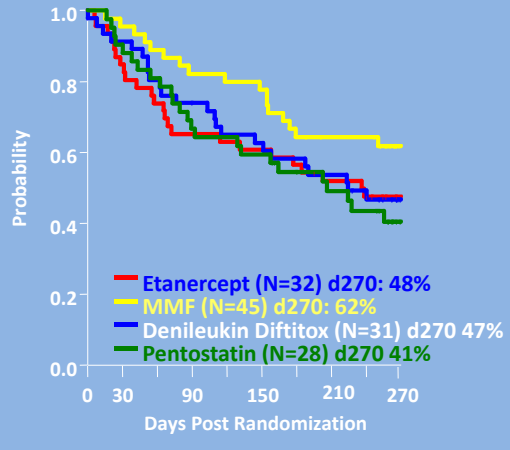
	Etanercept (N=46)	MMF (N=45)	Denil (N=47)	Pentostatin (N=42)
Complete Response	44%	73%	55%	62%
Complete Response (Excl. prior MMF)	53%	73%	61%	64%
Treatment Failure *	24%	9%	26%	29%

* Treatment Failure = no response, progression, or addition of another immunosuppressive agents by day 56.

Overall Survival



Relapse-Free Survival

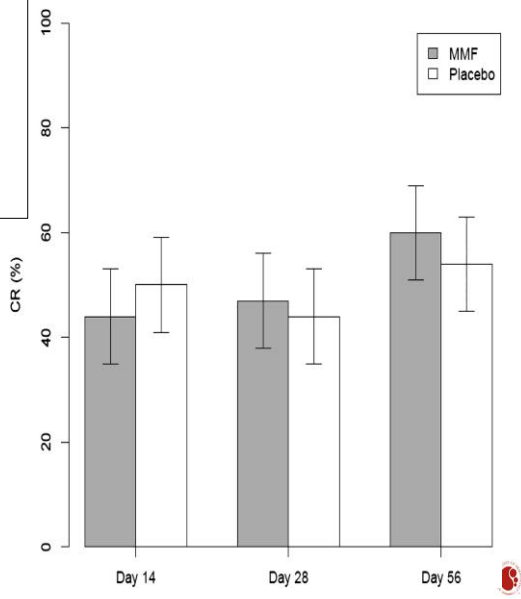


F/U Randomized Trial: 0802

MMF vs. Placebo + steroids for initial aGVHD therapy

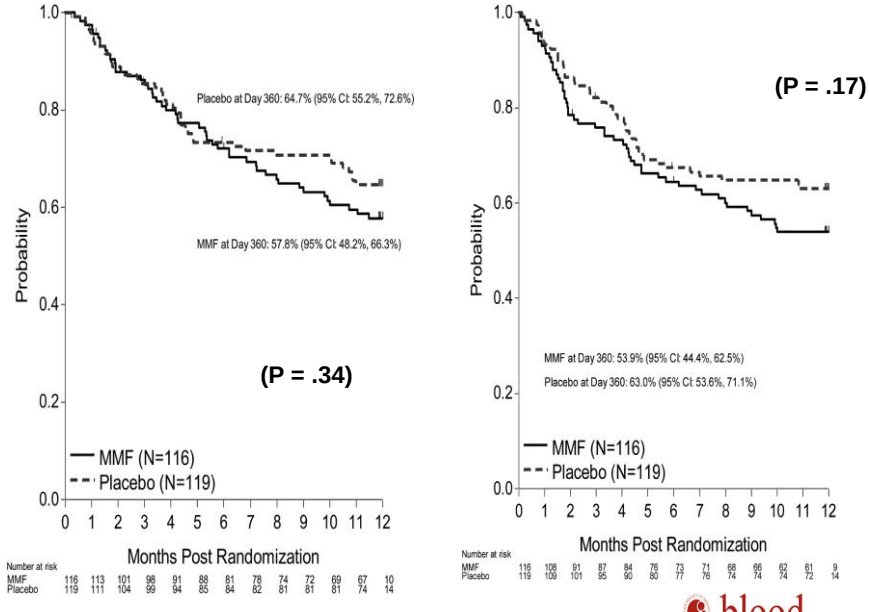
No advantage to adding MMF

Patients in CR over time.



Javier Bolaños-Meade et al. Blood 2014;124:3221-3227

Survival and DFS by study arm



GVHD Treatment: BMT CTN 0302/0802

- MMF + steroids seemed to provide a benefit in 0302; Not confirmed follow-up Phase III randomized trial (BMT CTN 0802)
- GVHD biomarker panels can be used for identification at high or low risk : biomarker panels may provide opportunities for early intervention and improved survival following HCT.

Next trials

- Distinguish high vs. low risk by clinical and biomarkers
- Testing Pred vs Sirolimus for low risk

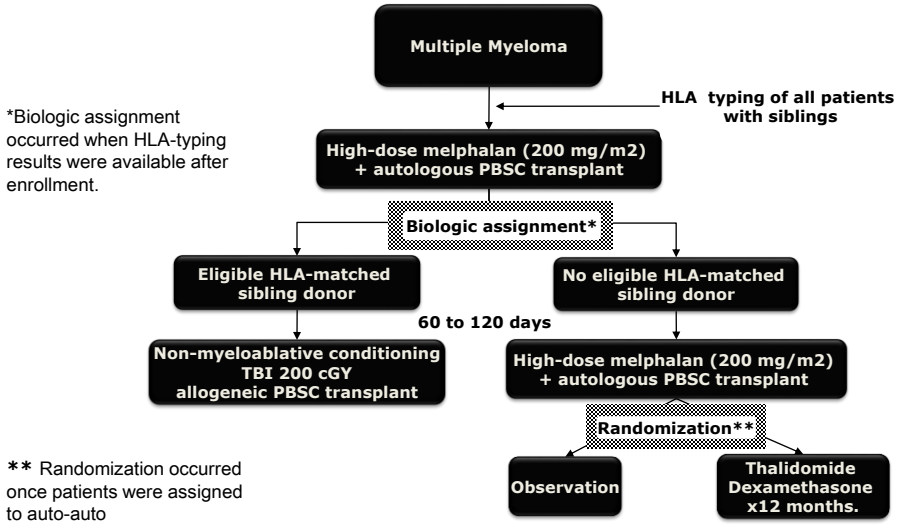
Multiple Myeloma

Auto/Auto vs. Auto/Allo transplantation

Post Auto maintenance

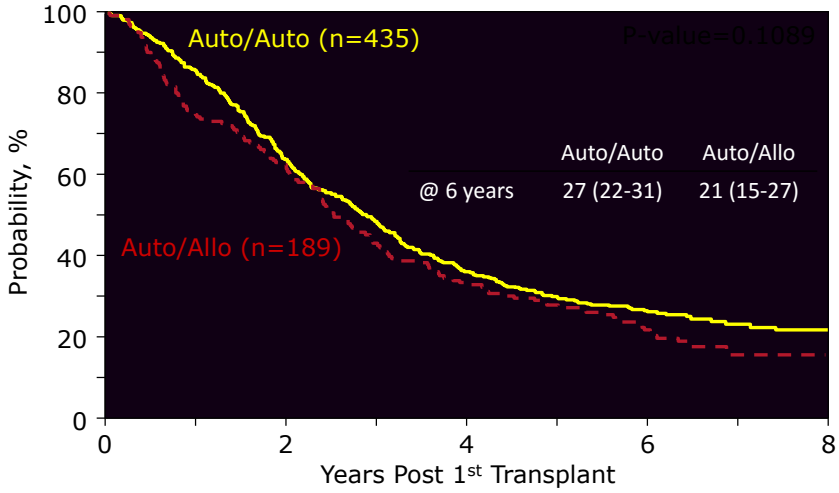
Post Auto strategies

BMT CTN 0102 Auto/Auto vs. Auto/Allo for myeloma

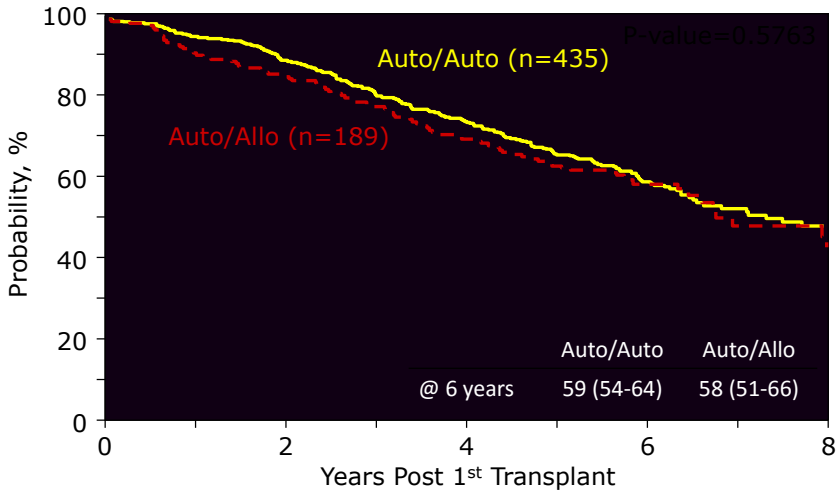


PRIMARY ENDPOINT : 3yr Progression Free Survival

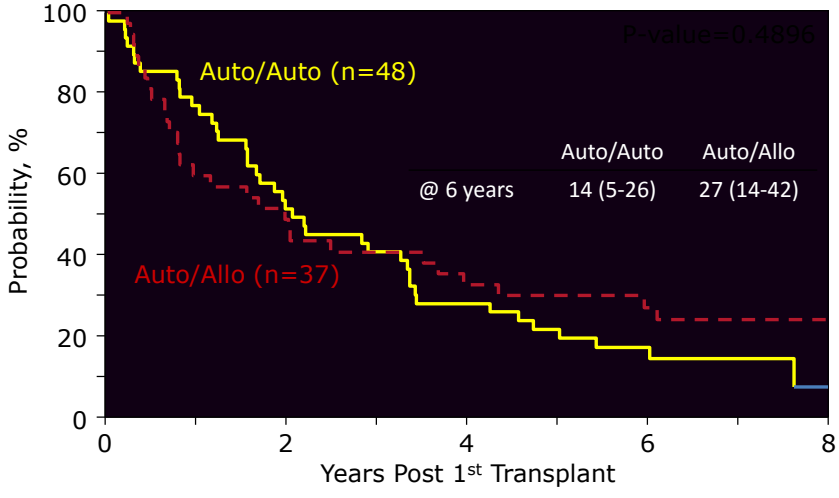
Progression-free Survival by Treatment Arm Standard Risk

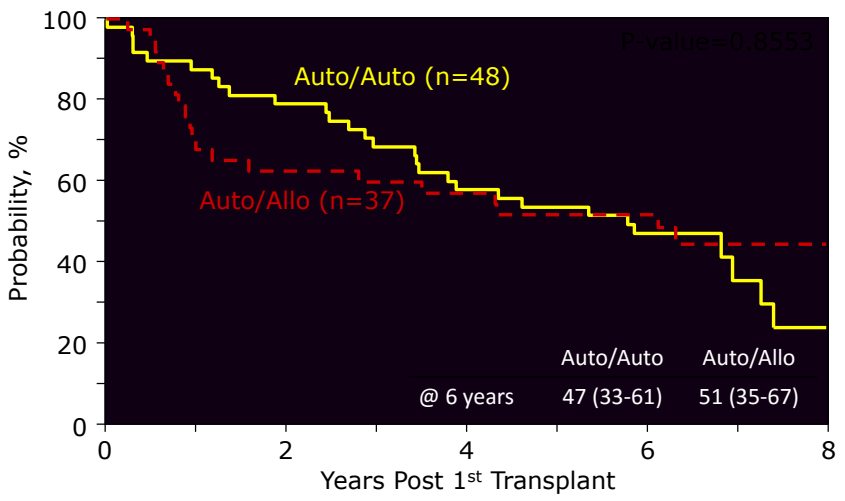


Overall Survival by Treatment Arm
Standard Risk



Progression-free Survival by Treatment Arm
High Risk



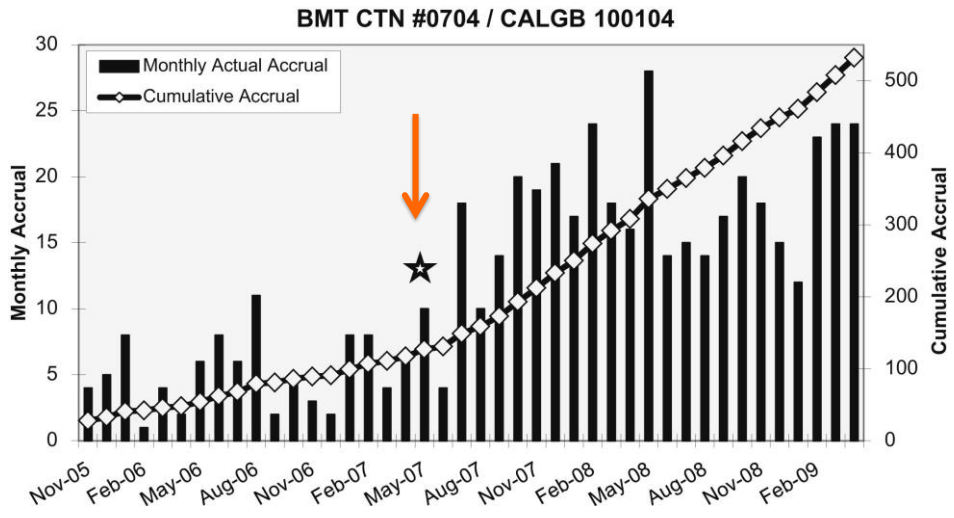


Multiple Myeloma: BMT CTN 0704

A Phase III, randomized, double-blind study maintenance therapy with
Lenalidomide or placebo
following autologous transplantation for Myeloma

- BMT CTN was an important contributor to this study, which was led by Cancer and Leukemia Group B (CALGB).

Network power: Accelerated enrollment after CTN joins in



ELSEVIER

Progression Free & Overall Survival

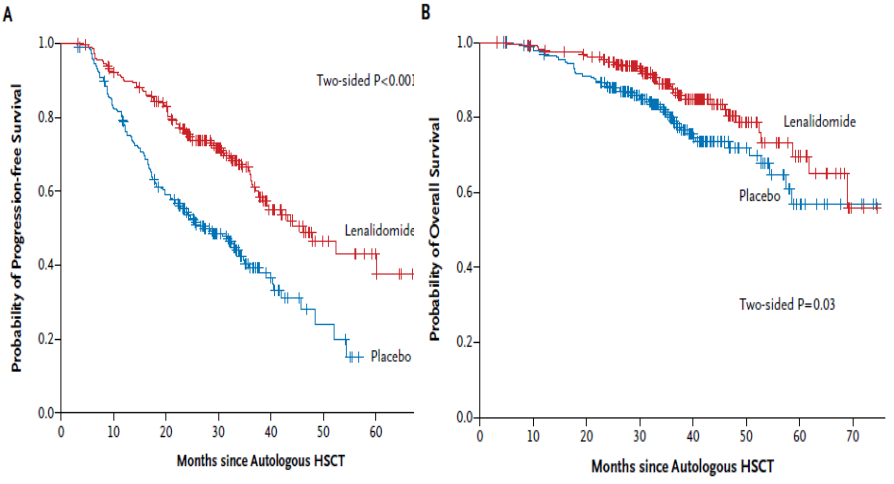


Figure 1. Kaplan–Meier Estimates of Progression-free and Overall Survival.
HSCT denotes hematopoietic stem-cell transplantation.

Multiple Myeloma: BMT CTN 0704

Maintenance therapy with Lenalidomide or placebo following autologous transplantation for Myeloma

- Lenalidomide maintenance therapy prolongs remission and survival after autologous HCT for multiple myeloma
- Major change in clinical practice, with most myeloma patients now receiving lenalidomide maintenance after HCT.

[***change of practice***]

McCarthy, NEJM

0702 Post Autograft strategies for Myeloma

Autologous HCT

then either:

Nothing; 2nd (Tandem) autograft; 4 cycles consolidation Chemotherapy [RVD]

All get Lenalidomide maintenance

Results coming soon

Multiple Myeloma

Auto/Auto vs. Auto/Allo transplantation

Post Auto maintenance

Post Auto strategies

Early vs. Late Autograft

Allotransplant + maintenance

Cellular vaccine post autotransplantation

Supportive Care: BMT CTN 0101

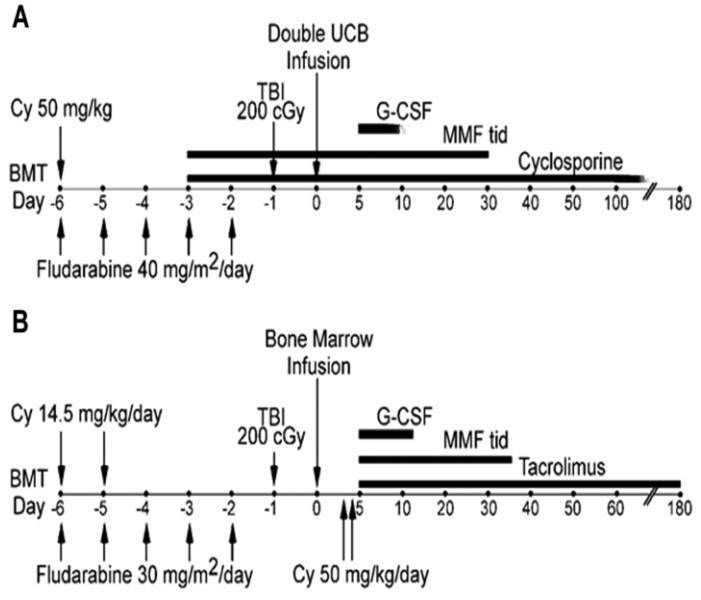
Fluconazole versus voriconazole for the prevention of invasive fungal infections in allogeneic HCT recipients

- Fluconazole, a low-cost antifungal agent, has similar efficacy as and is more cost-effective than the more expensive drug, voriconazole, in preventing serious fungal infections in the first six months after HCT. [***change of practice***]
- Demonstrated that voriconazole may be a cost-effective primary antifungal prophylaxis for a subset of patients undergoing an allogeneic HCT for AML.

Exploring New Graft Sources that Can Better Serve Minorities

- BMT CTN 0603 and 0604: Parallel Phase II study of reduced intensity HCT in adults:

- haploidentical bone marrow or
- unrelated donor umbilical cord blood

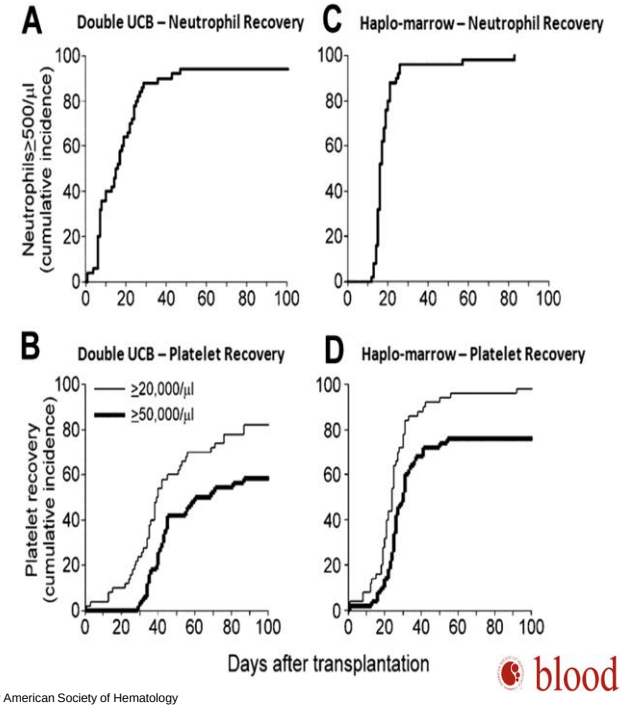


Brunstein 2011

blood

Neutrophil Recovery

Platelet recovery

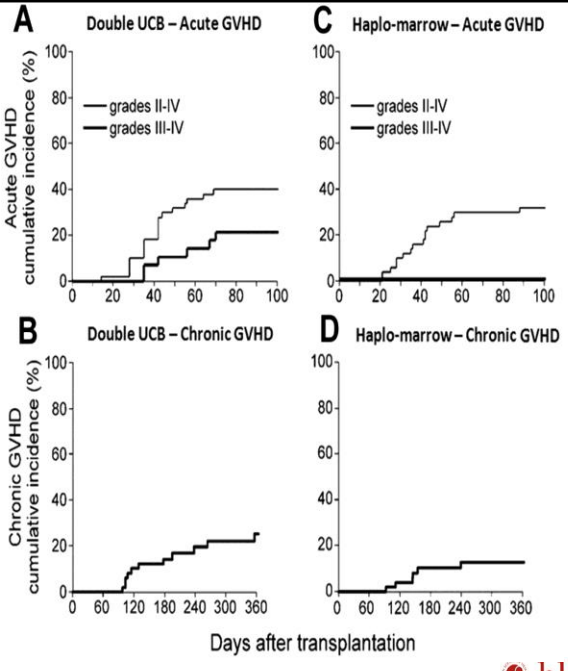


Brunstein 2011

blood

aGVHD

cGVHD



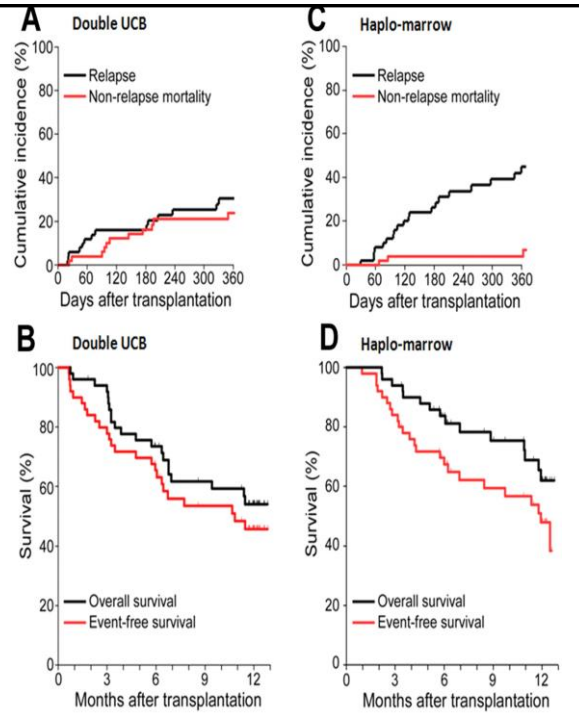
Brunstein 2011

blood

Relapse

TRM

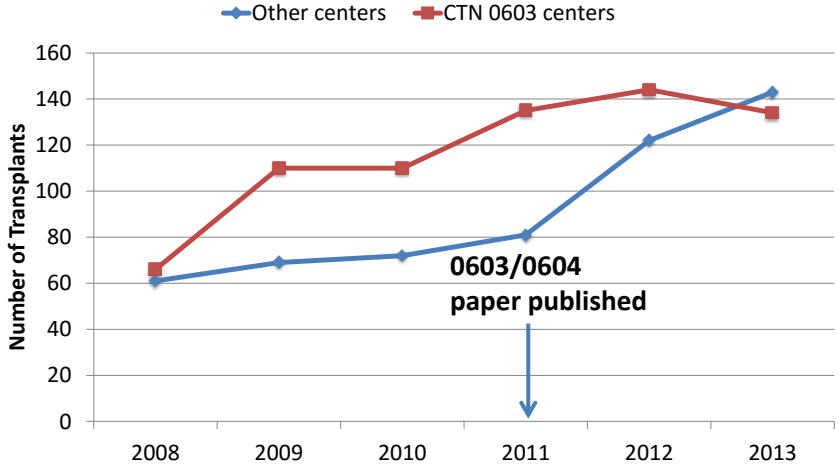
Survival



Brunstein 2011

blood

Haplo-Identical Transplantation for Hematologic Malignancy



Graft Sources: BMT CTN 0603/0604

- Reduced-intensity conditioning and haploidentical bone marrow transplantation or double UCB transplantation in adults with hematologic malignancies
- Acceptable outcomes with either double cord or haploidentical bone marrow

→Many more adults should be offered HCT, even if an HLA-matched adult donor is not available.
[***change of practice***]

- Haplo vs. UCB being compared in a randomized Phase III trial (BMT CTN 1101).

Is More Better

- Reduced Intensity Conditioning

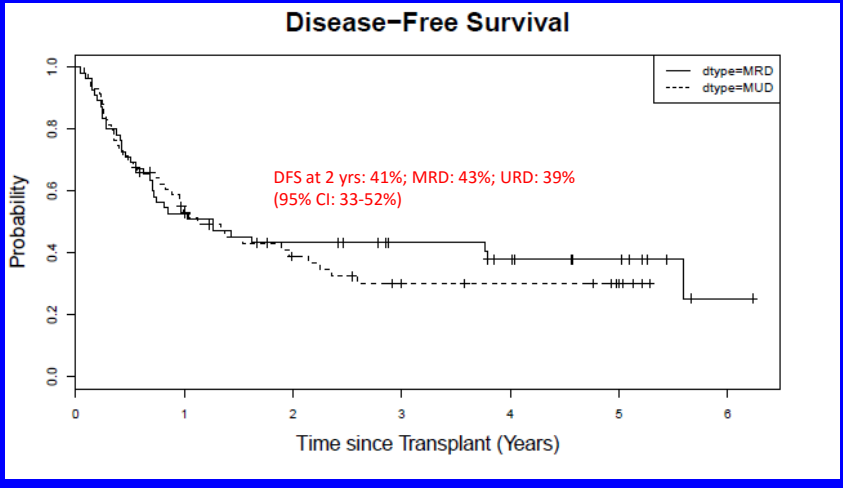
Less toxic

Suitable for Older or more frail patients

Conditioning Intensity in AML/MDS: High vs. Reduced Intensity Conditioning

- CALGB 100701/CTN 0502: Reduced intensity AlloBMT for elderly patients with AML
  - Would not have completed without BMT CTN

CALGB 100103/BMT CTN 0502
Disease Free Survival



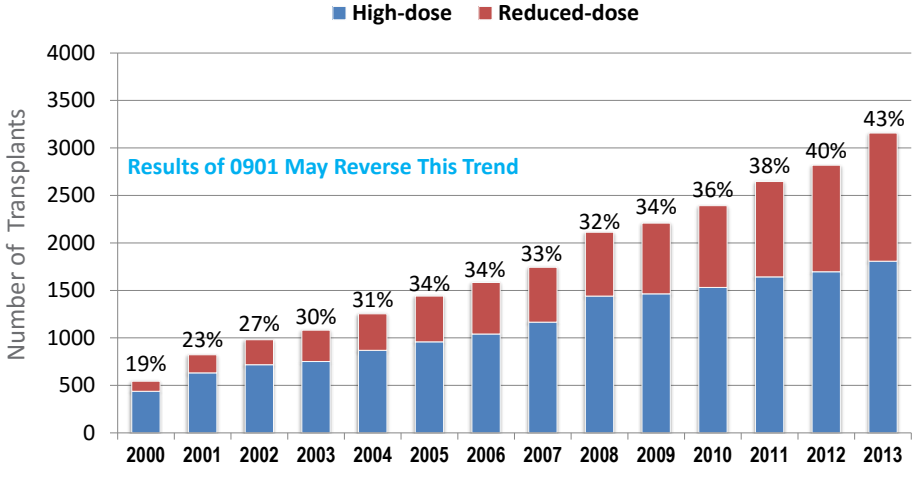
Median follow up: 4.9 yrs

Devine et al, JCO

BMT CTN 0901 Best Conditioning Regimen in AML/MDS: Randomized Trial of High vs. Reduced Intensity Conditioning

- **Study stopped after 272 of planned 356 patients enrolled (ahead of schedule) -- apparent outcome benefit in the high-dose arm**
- BMT CTN is the *only* way that this question could have been addressed

Increasing Use of Reduced Intensity Conditioning in Allogeneic Transplants in Adults with AML & MDS



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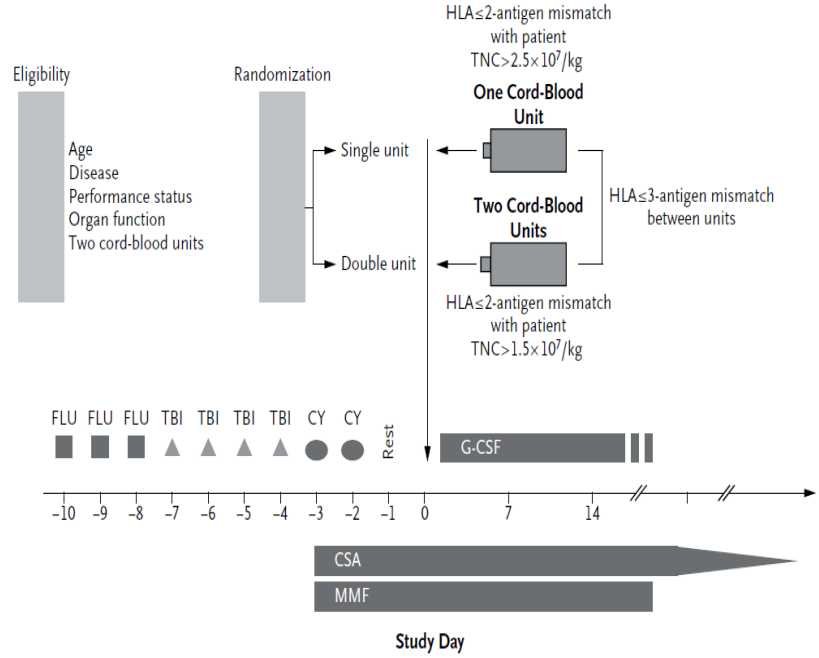
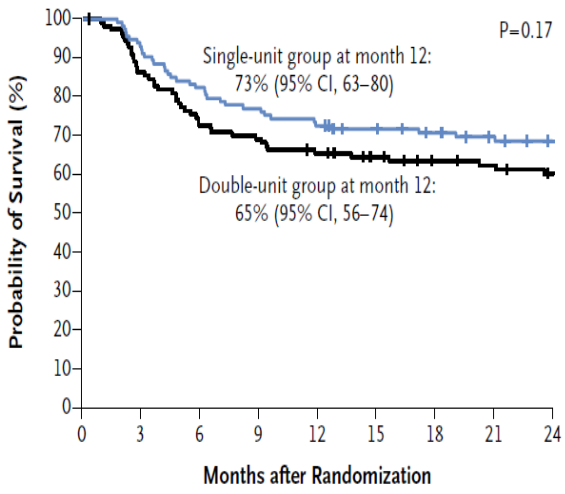
Challenging "conventional wisdom": is More better

- BMT CTN 0501: Randomized comparison of one vs. two cord blood units in children (collaboration with COG)
  - Only randomized trial in cord blood transplantation - ever
  - Accrued 224 patients *on time* (1 yr survival endpoint)

Wagner, NEJM, 2014



Single vs Double UCB grafts in Children

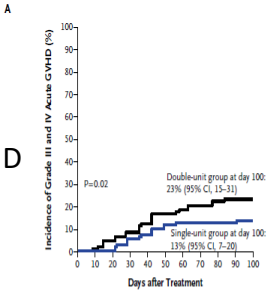


No. at Risk

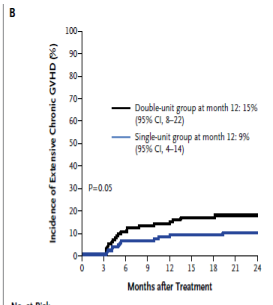
Double-unit group	111	95	80	76	71	64	59	57	54
Single-unit group	113	103	93	87	82	75	71	66	63

Survival: similar in both groups

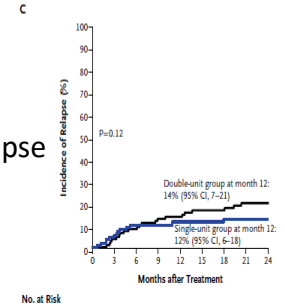
III-IV aGVHD



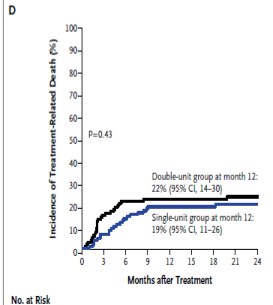
cGVHD



Relapse



Treatment related mortality



Major Complications

Challenging "conventional wisdom": More is not better

- BMT CTN 0501: Randomized comparison of one vs. two cord blood units in children (collaboration with COG)
  - **Similar survival with two versus one unit:**
  - **Two unit transplants were associated with more GVHD and slower platelet recovery**
  - **Important implications for practice/ public policy**

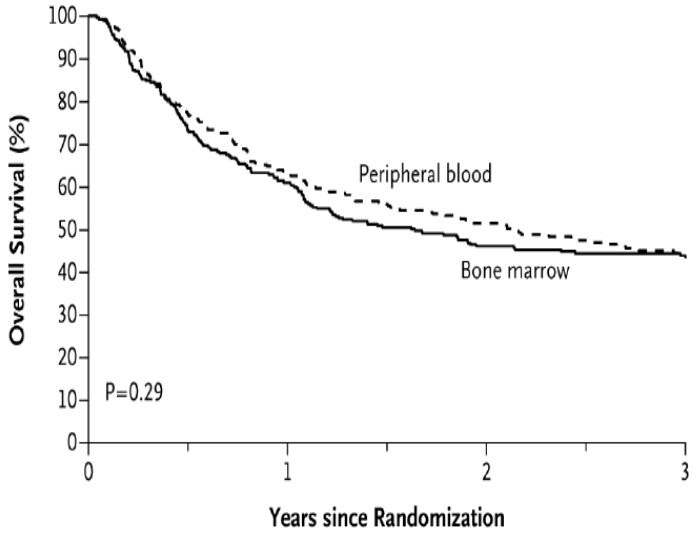
Wagner, NEJM, 2014

Challenging "conventional wisdom": Bone Marrow vs Peripheral Blood

- **BMT CTN 0201**: Compared bone marrow vs. peripheral blood for unrelated donor transplantation
  - Largest study of unrelated donor transplantation ever done
  - Close collaboration with National Marrow Donor Program

Anasetti NEJM 2012

Survival with BM vs PBSC in URD Transplantation



Challenging "conventional wisdom": Bone Marrow vs Peripheral Blood

- **BMT CTN 0201**: Compared bone marrow vs. peripheral blood for unrelated donor transplantation
  - Results challenged the conventional wisdom that peripheral blood stem cells are better (used for ~70% of transplants)
    - **No survival benefit with peripheral blood**
    - **More chronic GVHD with PBSC requiring prolonged immune suppression**

Anasetti NEJM 2012

Network Challenges

- Pick the best questions
- Address those requiring multicenter participation and more accrual
- Test approaches that can change the field
- Add correlative studies to inform the next trials

Five Year Results of BMT CTN 0201

Clinical Implications

No relevant conflicts of interest

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

Background

- BMT CTN 0201 was a RCT of unrelated donor bone marrow (BM) vs. peripheral blood (PB) transplantation for hematologic malignancies
- Results showed similar survival, DFS, TRM
- BM had a higher rate of graft failure (9% vs. 3%, p=0.002)
- PB had a higher rate of chronic GVHD (53% vs. 41%, p=0.01)

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

Anasetti C et al, NEJM 2012; 367:1487

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Parent Trial Eligibility Criteria

- Age up to 66 years
- First transplant
- Acute and chronic leukemia, MDS, MF
- 5/6 or 6/6 match at HLA-A, B, DRB1
  - 98% 7/8 or 8/8 matched
- No active infection

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Parent Trial Study Design

- Four myeloablative/RIC regimens allowed
  - Cyclophosphamide/TBI
  - Cyclophosphamide/Busulfan
  - Fludarabine/Busulfan/ATG
  - Fludarabine/Melphalan
- Two GVHD prophylaxis regimens
  - Cyclosporine/methotrexate +/- others
  - Tacrolimus/methotrexate +/- others
- More than 80% of similar transplants use PB

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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QOL was a secondary endpoint

- Stephanie J. Lee
- Brent Logan
- Peter Westervelt
- Corey Cutler
- Ann Woolfrey
- Shakila P. Khan
- Edmund Waller
- Richard Maziarz
- Juan Wu
- Bronwen Shaw
- Dennis Confer
- Mary Horowitz
- Claudio Anasetti

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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QOL Eligibility Criteria/Study Design

- Age >16
- English or Spanish speaking
- Patient-reported outcomes (PROs) collected prior to randomization and at 0.5, 1, 2 and 5 years after transplantation
  - FACT-BMT
  - MHI (mental health inventory)
  - Lee chronic GVHD symptom scale (post-HCT only)
  - Occupational functioning

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Study Design cont.

- Data collection centralized
  - One organization collected baseline, 0.5, 1, 2 years
  - NMDP/Be The Match collected 5 year assessments
- Response rates, for surviving patients
  - Baseline (n=368) – 72%
  - 0.5 year (n=146) – 40%
  - 1 year (n=123) – 41%
  - 2 year (n=71) – 29%
  - 5 year (n=148) – 76%

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Responder characteristics

	Enrollment, pre-HCT			5 year assessment		
	Responder	Non-responder	p-value	Responder	Non-responder	p-value
High risk disease, n (%)	102 (25.8)	47 (38.5)	0.007	24 (15.8)	14 (32.6)	0.014
Karnofsky score			<0.001			0.82
≥90%, n (%)	245 (62.0)	55 (45.1)		101 (66.4)	31 (72.1)	
<90%, n (%)	108 (27.3)	32 (26.2)		39 (25.7)	9 (20.9)	
Missing	42 (10.6)	35 (28.7)		12 (7.9)	3 (7.0)	
Age ≥ 40, n (%)	245 (62.0)	72 (59.0)	0.55	89 (58.6)	15 (34.9)	0.006

No difference in graft source, diagnosis, sex, race, conditioning regimen, GVHD prophylaxis, HLA mismatching

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Five year results of BM vs. PB

QOL scale	Bone marrow (n=102)	Peripheral blood (n=93)	P value	Clinically significant difference[1]	Difference between BM and PB (95% CI)[2]
FACT-BMT TOI (↑ better) Mean +/- SE	76.7 +/- 1.6 (n=79)	70.5 +/- 1.9 (n=69)	0.014	8.5	6.2 (1.3-11.1)
MHI – Psychological well-being (↑ better) Mean +/- SE	78.9 +/- 1.7 (n=80)	72.2 +/- 1.9 (n=72)	0.011	8.4	6.7 (1.6-11.8)
MHI-Psychological Distress (↓ better) Mean +/- SE	16.0 +/- 1.3 (n=80)	19.0 +/- 1.5 (n=71)	0.128	6.5	-3.0 (-6.8,0.9)
Chronic GVHD symptoms (↓better) Mean +/- SE	13.1 +/- 1.5 (n=80)	19.3 +/- 1.6 (n=72)	0.004	7.1	-6.3 (-10.5, -2.0)

FACT-BMT TOI, Functional Assessment of Cancer Therapy, Bone Marrow Transplant Trial Outcome Index; MHI, Mental Health Inventory; GVHD, Graft-versus-Host Disease; SE, standard error
[1]0.5 x STD
[2]Adjusted for enrollment values and missing data using inverse probability weighting using significant clinical characteristics

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

Lee et al, JAMA Onc 2016, in press

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Baseline predictors of 5 year PROs

	Variable	Level	Mean/Slope	Standard error	p-value
MHI – Psych well-being (↑better)	Graft type	Bone marrow Peripheral blood	78.0 71.7	1.8 2.0	0.015
	Age	≤ 30 years 31-50 years >50 years	77.2 69.6 77.8	3.3 2.5 1.6	0.02
	Baseline MHI Psych well-being	Slope	0.4	0.09	<0.001
Chronic GVHD symptoms (↓ better)	Graft type	Bone marrow Peripheral blood	14.4 20.5	1.5 1.6	0.002
	Age	≤ 30 years 31-50 years >50 years	13.7 23.1 15.5	2.6 1.9 1.4	0.002
	Gender	Female Male	21.5 13.4	1.6 1.5	<0.001
	Primary disease	AML ALL CML MDS	15.5 16.8 23.4 14.1	1.5 2.5 2.5 2.3	0.019

Additional results – chronic GVHD

	BM	PB	P-value
Chronic GVHD – skin (0-100, ↓ better) Mean +/- SE	10.8 +/- 1.8 (n=80)	16.2 +/- 2.3 (n=72)	0.06
Chronic GVHD – eyes (0-100, ↓ better) Mean +/- SE	21.0 +/- 3.0 (n=80)	44.3 +/- 4.1 (n=72)	<0.001
Chronic GVHD – mouth (0-100, ↓ better) Mean +/- SE	6.7 +/- 2.1 (n=80)	9.2 +/- 1.7 (n=72)	0.09
Chronic GVHD – lung (0-100, ↓ better) Mean +/- SE	3.8 +/- 0.9 (n=80)	9.2 +/- 1.7 (n=72)	0.004
Chronic GVHD – nutrition (0-100, ↓ better) Mean +/- SE	3.3 +/- 0.8 (n=80)	5.3 +/- 1.2 (n=72)	0.12
Chronic GVHD – energy (0-100, ↓ better) Mean +/- SE	25.5 +/- 2.7 (n=80)	37.6 +/- 3.1 (n=72)	0.003
Chronic GVHD – psych (0-100, ↓ better) Mean +/- SE	20.1 +/- 3.0 (n=80)	23.3 +/- 2.8 (n=72)	0.45

CLINICAL TRIALS NETWORK

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Additional 5 yr results – reported by centers

	BM (n=102)	PB (n=93)	P-value
Chronic GVHD, n (%)			0.03
No cGVHD	72 (71)	46 (49)	
Mild	17 (17)	21 (23)	
Moderate	9 (9)	16 (17)	
Severe	4 (4)	8 (9)	
Missing	0	2 (2)	
Skin sclerosis, n (%)	8 (8)	17 (18)	0.03
Eye involvement, n (%)	15 (15)	31 (33)	0.002
Musculoskeletal involvement, n (%)	3 (3)	14 (15)	0.003
Avascular necrosis, n (%)	5 (5)	14 (15)	0.02

No differences in:
- mouth, lung or GI chronic GVHD involvement
- diabetes, dialysis, hypothyroidism, cardiac

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Return to work

- Likelihood of return to full or part time work outside the home was higher for BM
  - OR 1.5, 95% CI 1.2-2.0, p=0.002
  - Adjusted for work status before transplant
  - Missing data adjusted for based on graft source, disease risk, and age

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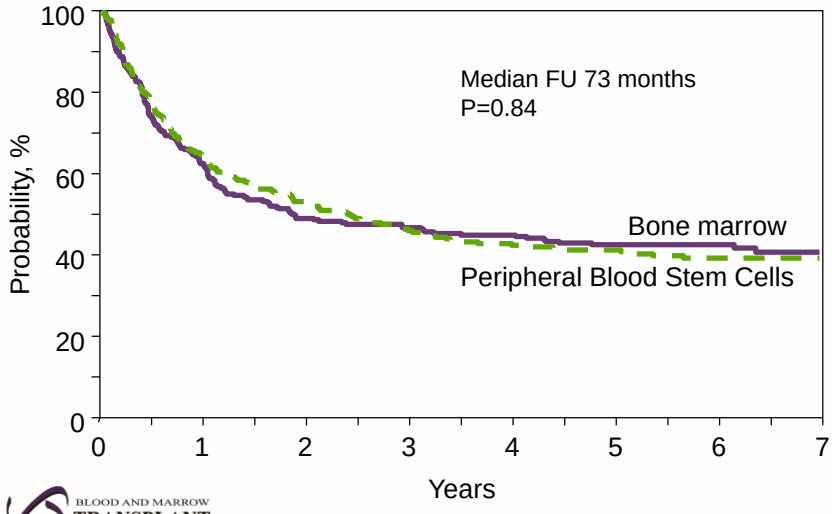
Overall Survival



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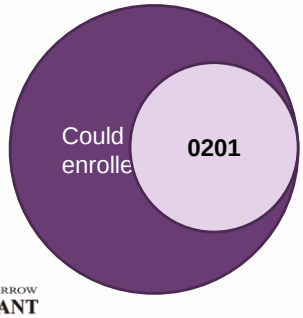
Generalizability

- Compared characteristics and outcomes of 0201 trial participants with non-participants
- Held constant: centers, time period, conditioning regimens and GVHD prophylaxis



Khera N et al, BBMT 2015; 21: 1815

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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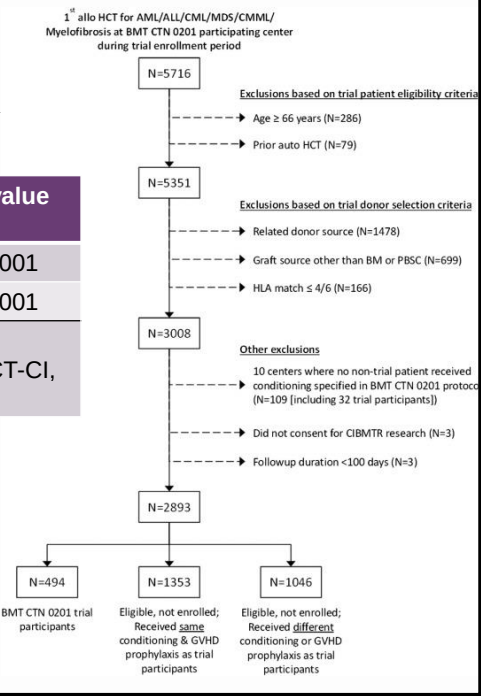
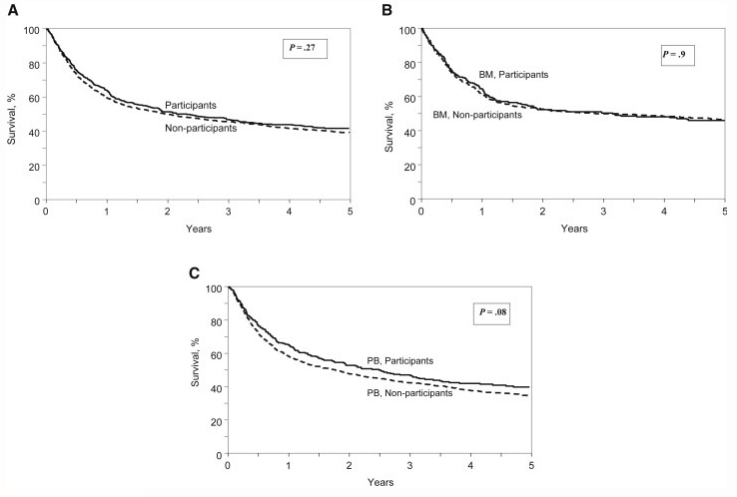
Study population

	0201 vs. Non-participants	P-value
BM	50% vs. 34%	<0.001
ATG	26% vs. 32%	<0.001
Not different: Pt age, sex, disease, race/ethnicity, HCT-CI, HLA match, time from dx to HCT		



BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

Results



BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Conclusions

- At 5 years after HCT, recipients of unrelated donor BM, compared with PB, have:
  - Similar survival, relapse, TRM (generalizable)
  - Better psychological well-being
  - Less burdensome chronic GVHD symptoms
  - Are 50% more likely to go back to work
- No outcome for which PB was better
- PB is still used for >80% of unrelated donor transplants for similar patients
- Will more bone marrow be used?

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Limitations/Implementation Concerns

- Newer GVHD prophylaxis regimens not represented
  - Clinical trials may require PB
- BM is harder to arrange and more difficult to reschedule than PB
- Lingering concerns about engraftment and relapse with BM: large pt/small donor, transit time, disease type and status
- Donor recovery longer with BM, although with time they achieve similar recovery to PB

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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Case

- 40 y/o with high risk AML in CR1
- Myeloablative conditioning
- Standard tacrolimus/MTX GVHD prophylaxis
- 8/8 young unrelated donor

BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

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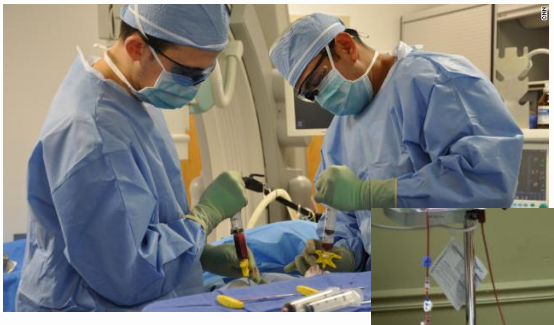
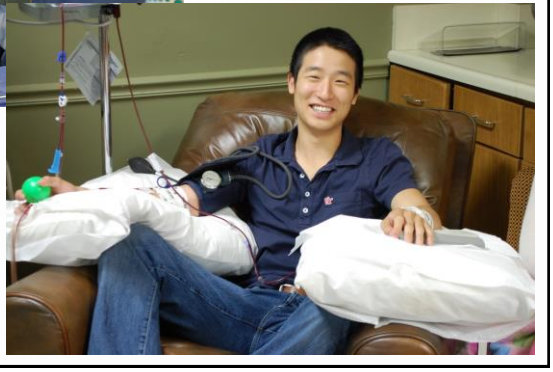
What graft source would you prescribe?





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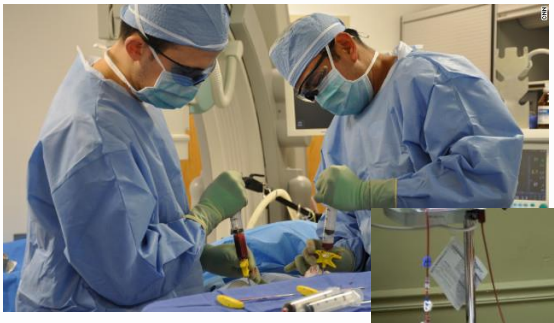
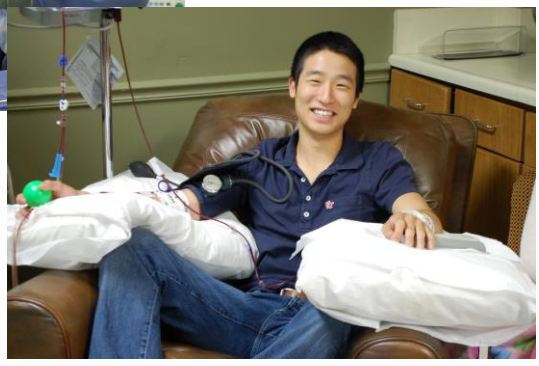
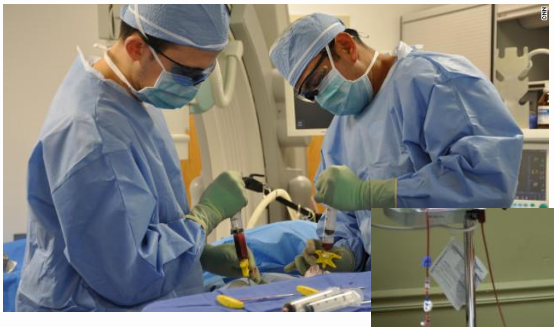
What graft source would you prescribe? donate

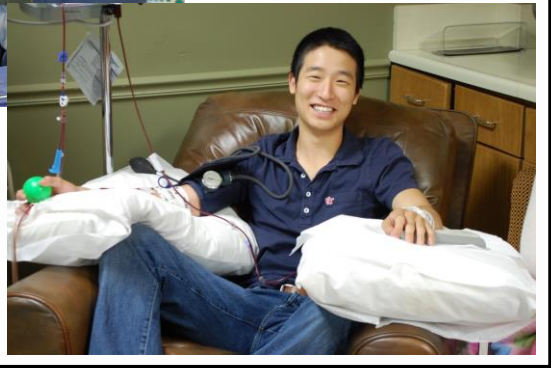




BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

What graft source would you prescribe? want





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