

BSBMT Indications for BMT

Abbreviations:

S = standard of care

CO = clinical option, can be considered after assessment of risks and benefits

D = developmental, further trials are needed

GNR = generally not recommended

Section 1

CML

	Sibling Allograft	Unrelated donor transplant	Autologous transplant
Chronic phase -TKI refractory ¹ (after trial of at least 2 TKIs)	S ^{1,2,3}	S ^{1,2,3}	GNR
-TKI intolerant (Grade 2+ toxicity to at least 2 TKIs)	S ¹	S ¹	GNR
-T315I mutation	S ¹	S ¹	GNR
Accelerated phase -after initial therapy with TKI	S ^{4,5}	S ^{4,5}	GNR
Blast crisis	GNR	GNR	GNR
2nd chronic phase	S ^{4,6}	S ^{4,6}	D ⁷ (if Ph –ve cells have been stored)

¹ For definition see Baccharani et al

¹Baccharani et al, 2009, J Clin Onc 27: 6041-6051

² Lee et al, Blood 2008, 112: 3500-3507

³ Bacher et al, Ann Haematol 2009, 88: 1237-1247

⁴ Saussale et al, Blood 2010 115: 1880-1885

⁵ Jiang et al, Blood 2011, 117: 3032-40

⁶ Weisser et al, Leuk Lymphoma 2007, 48: 295-301

⁷. Bhatia et al, Haem/Onc Clin North Am 2004, 18 : 715-732

Myeloma

	Sibling transplant [‡]	MUD transplant	First Autograft	Second Autograft
First Line	S ^{16, 17}	CO ¹⁸ -Selected patients or as part of clinical trial	S ⁹ -for patients suitable for intensive treatment	CO ^{10,11} (Tandem autograft may be considered if no CR after 1st autograft)
Relapse	CO ^{12, 19}	CO -Selected patients or as part of clinical trial	S (If not done in first response but patient is considered fit)	S ¹³ -If time to re-treatment after 1st autograft >18m or as part of NCRN Myeloma X trial
Plasma cell leukaemia	S ¹⁵ -If chemo responsive disease -Selected young patients <55 years	CO ¹⁵ -If chemo responsive disease	S ¹⁵ -If no suitable donor or unfit for allograft	CO

[‡] - Suitability for a myeloablative versus reduced-intensity is based on biological suitability (age, co morbidity, advanced disease stage, etc)

References:

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| <p>8. Gahrton et al, Haematologica 2007, 92: 1513-8</p> <p>9. Child et al, New Engl J Med 2003, 348: 1875-8</p> <p>10. Abdellcefi et al, Blood 2007, e-pun Nov 8</p> <p>11. Cavo et a, J Clin Onc 2007, 25: 2434-41</p> <p>12. Elice et al, Am J Haematol 2006, 81: 426-31</p> <p>13. Alvares et al, Haematologica 2006, 91: 141-2</p> | <p>14. Perfetti et al, Haematologica 2006, 91: 1635-43</p> <p>15. Saccaro et al, Am J Haematol, 2005, 78: 288-94</p> <p>16. Levenga H et al, Biol Blood Marrow Transplant, 2010 Mar: 16(3): 320-32</p> <p>17. Rotta et al, Blood, 2009 Apr 2: 113 (14): 3383-91</p> <p>18. Kroeger et al, Br J Haematol, 2010 Jan; 148(2): 323-31</p> <p>19. Efebera YA et al, Biol Blood Marrow Transplant, 2010 Feb 20</p> |
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Other Plasma Cell Dyscrasias

	Sibling transplant	MUD transplant	First Autograft	Second Autograft
AL amyloid	GNR	GNR	CO ¹⁴ -As per risk-adapted protocol	GNR
POEMS	GNR	GNR	CO ¹⁶	CO ¹³ -If time to re-treatment after 1st autograft >18m or as part of a clinical study

References:

13. Jaccard et al, Blood 2002, 99: 3055-9

14. Perfetti et al, Haematologica 2006, 91: 1635-43

General Comments

1. Generally RIC transplants are performed for patients >45-50 years of age or for patients with significant co-morbidities using the HSCT co-morbidity index. In the context of certain clinical trials the age for choosing a RIC transplant may be lower. Patients with a score >3 are generally not suitable for any HSCT
2. For unrelated donor transplants usually either a full 10/10 match at HLA A, B, C and DR is required or a single mismatch
3. Cord Blood transplants are an alternative for patients lacking a sibling or unrelated donor (as defined above). Usually these patients are from ethnic minority.

Section 2

AML

		Sibling transplant	MUD transplant	Autograft	Comments
APL CR1 APL CR2 PCR+		GNR S	GNR S	GNR GNR	BCSH guidelines
APL CR2 PCR-		CO	GNR	S	
AML -good risk	CR1 CR2	GNR S	GNR S	GNR CO	BCSH guidelines AML15/16 trial protocols
AML -standard risk	CR1	S	S	GNR	AML 15/16 protocols
	CR2	S	S	CO	
AML -poor risk*	CR1	S	S	GNR	AML 15/16 protocols
	CR2	S	S	CO	
AML not in remission		CO	CO	GNR	Fung et al ¹ , Cook et al ²

* Poor risk defined as either 1. cytogenetics (MRC criteria), 2. Secondary or therapy – related AML, 3. Failure to achieve CR with standard AML induction therapy

References

1. Fung HC, Stein A, Slovak M, et al. A long-term follow-up report on allogeneic stem cell transplantation for patients with primary refractory acute myelogenous leukemia: impact of cytogenetic characteristics on transplantation outcome. Biol Blood Marrow Transplant. 2003;9:766771
2. Cook G, Clark RE, Crawley C, et al. The outcome of sibling and unrelated donor allogeneic stem cell transplantation in adult patients with cute myeloid leukemia in first remission that were initially refractory to first induction chemotherapy. Biol Blood Marrow Transplant. 2006;12:293-300

Section 3

ALL

	Sibling transplant	MUD transplant	Autograft
CR1			
-standard risk	S ¹	GNR	GNR
-poor risk	S ¹	CO ²	GNR
CR2	S	S	GNR ³
Not in remission	GNR	GNR	GNR
Philadelphia positive ALL	S	S	GNR

References

1. Rowe et al. Blood 2006 (ASH plenary session)108:127, abstract no 2
2. Rowe and Goldstone Blood 110:2268-2275, 2007. Poor risk is defined as adverse cytogenetics, T-ALL with WCC>100, B-ALL with WCC>30, MRD positive after phase 2. Ideally this should be discussed with a member of the NCRI ALL group
3. Autografts, although inferior to chemotherapy in CR1 patients and inferior to allografts in CR2 patients may be justified when all other therapeutic options have been explored or the optimal therapy (eg chemotherapy) cannot be delivered

Section 4

BSBMT Indications For Haematopoietic Stem Cell Transplantation In Lymphoma

General Comments

- a. An allogeneic stem cell transplant may be considered in any disease category where autologous stem cell harvesting has failed.
- b. A MUD should be a 10/10, 8/8 or 9/10 allelic level match.

Hodgkin's Disease

	Autograft	Sibling transplant	MUD transplant
CR1	GNR	GNR	GNR
CR>1	S ¹	CO ²	CO ²
Relapse/ Primary Refractory -Chemosensitive -Chemorefractory	S ¹ CO	CO ² CO ²	CO ² CO ²
Relapse post autograft	GNR	CO ³	CO ³

References

- 1. Linch et al Lancet 1993; 341: 1050-1054, Schmitz et al Lancet 2002; 359: 2065-2071
- 2. Patients considered at high risk of failing an auto in CR1 eg CR1<1 year, PET⁺ post salvage, less than PR post salvage, chemorefractory
- 3. Peggs Lancet 2005; 365: 1906-1908., Sureda JCO 2008; 26: 455-462

Mantle Cell Lymphoma

	Autograft	Sibling transplant	MUD transplant
CR1/PR1	S ¹	CO ²	CO ²
CR/PR>1	S ¹	CO ²	CO ²
Chemorefractory	GNR	D	D
Relapse post autograft	GNR	CO ³	CO ³

References

1. Dreyling Blood 2005; 105:2677–2684
2. Khouri JCO 2003, Maris Blood 2004; 104: 3535, proposed NCRN trial (Rule et al)
3. Robinson Blood 2004; 104: 2322, Faulkner Blood 2004; 103: 428 -434

Follicular Lymphoma

	Autograft	Sibling transplant	MUD transplant
CR1/PR1	GNR ¹	GNR	GNR
CR/PR>1	S ²	CO ³	CO ³
Chemorefractory	GNR	D	D
Relapse post autograft	GNR	CO ⁴	CO ⁴

References

1. Lenz Blood 2004; 104: 2667-2674
2. Schouten JCO 2003; 21: 3918-3927
3. van Besien Blood 1998; 92: 1832-1836, Morris Blood 2004; 104: 3865-3871, Robinson Blood 2002; 100: 4310-4316, Faulkner Blood 2004; 103; 428-434
4. Morris Blood 2004; 104: 3865-387, Robinson Blood 2002; 100: 4310-4316

DLBCL

	Autograft	Sibling transplant	MUD transplant
CR1	GNR ¹	GNR	GNR
PR1 (sensitive to salvage)	CO	CO	CO
CR, PR>1	S ²	CO ³	CO ⁴
Chemorefractory	GNR	D	D
Relapse post autograft	GNR	CO ⁴	CO ⁴

References

1. Cochrane database
2. Philip NEJM 1995
3. Chopra JCO 1992; 10: 1690-1695, Bierman JCO 2003; 21: 3744-3753
4. Morris Blood 2004; 104: 3865-387

Peripheral T cell Lymphoma

	Autograft	Sibling transplant	MUD transplant
CR1	CO ¹	CO ²	CO ²
PR1 (sensitive to salvage_	CO ¹	CO ²	CO ²
CR/PR>1	S	CO	CO
Chemorefractory	GNR	D	D
Relapse post autograft	GNR	CO ²	CO ²

References

1. Reimer Blood 2005; 106:2074
2. Corradini JCO 2004; 22:2172-2176, Wulf BMT 2005; 36:271-273

Section 5

CLL

	RIC Sib allograft (1)	RIC VUD	Auto	UCB
Very high risk CR1 (2)	S	S	GNR	CT
High risk CR2(3)	S	S	CT	CT
Others CR >2 (4)	CO	CO	CO	CT
Richters transformation CR1	S	S	GNR	CT
T-PLL	S	S	CO	CT
B-PLL (5)	CO	CO	CO	CT

Notes

1. For most CLL patients, reduced intensity (RIC) conditioning is recommended however for some younger patients (<45 years) with very high risk disease and a matched sibling donor then standard intensity conditioning may be preferable (CO).
2. Very high risk CLL defined as CLL with >20% cells showing del. 17p or purine analogue refractory. These patients should be treated with p53 independent therapy, such as high dose methyl prednisolone and/or alemtuzumab to maximum response and then allografted if possible in CR1
3. High risk CLL defined according to EBMT criteria:¹
 - i. Relapse within 6 months of PA therapy
 - ii. Relapse within 2 years of intensive therapy including PA/alkylator combinations, chemo-immunotherapy or autologous transplantation
4. Other indications. Includes patients not fulfilling criteria 2 or 3 who are in second or subsequent relapse with at least one other commonly recognised adverse features listed below:
 - i. Bone marrow failure according to Binet criteria
 - ii. Unmutated Vh genes (<98% germline or Vh3.21)
 - iii. ZAP 70+ (>20%)
 - iv. CD38+ (>7%)
 - v. Del 11q or trisomy 12
5. Approx 20% of cases of B-PLL actually mantle cell lymphoma and should be treated accordingly. B-PLL otherwise rare and should be treated on a case by case basis (CO)

Abbreviations

S – Standard of care

CO – Clinical opinion

GNR – Generally not recommended

CT – Only in context of clinical trial

CR1 or CR2 – Defined as first or second best response to therapy and includes either complete or partial remission as defined in NCI response criteria². Patients with stable or progressive disease may respond to allogeneic transplantation but should be considered on a case by case basis (CO)

References

1. Dreger P, Corradini P, Kimby E, et al. Indications for allogeneic stem cell transplantation in chronic lymphocytic leukemia: the EBMT transplant consensus. *Leukemia*. 2007;21:12-17'
2. Cheson BD, Bennett JM, Grever M, et al. National Cancer Institute-sponsored Working Group guidelines for chronic lymphocytic leukemia: doi:10.1182/blood-2007-06-093906 Prepublished online Jan 23, 2008

Section 6

Indications for allograft in adult patients with aplastic anaemia

Aplastic anaemia

	Matched sibling	MUD	UCBT	Autologous
Severe AA (SAA) < 50 yr	S	S if failed IST and no sibling	CO	GNR
SAA >50 yr	S if failed IST	s if failed IST and no sibling	D	GNR
Constitutional AA	S	S if no sibling	CO	GNR

IST = failed at least one course of IST (immunosuppressive therapy)

References

1. BCSH guidelines Brit. J. Haem. 2009; 147: 43
2. Bacigalupo et al. BBMT 2009; 15; issue 2, 5
3. Bacigalupo et al. Haematologica 2010 in press
4. Maury et al. Haematologica 2009; 94: 1312
5. Bacigalupo. EBMT data presented at ASBMT/Tandem Meeting 2010
6. Young NS, Bacigalupo A, Marsh J. BBMT 2010; 16; issue 1, S119

Section 7

Indications for Transplantation for Adults with Myelodysplastic Syndromes

MDS

IPSS score	Autograft	Sibling Allograft	VUD allograft	UCBT
Low-Int-1	GNR	CO*	CO*	D**
Int-2, High	GNR	S	S	D**
t-MDS	GNR	S	S	D**

t-MDS: therapy related MDS

Reduced intensity conditioning protocols are recommended for patients aged 40-45 years or older, or in patients with pre-existing co-morbidities as defined using the HSCT co-morbidity index (HCT-CI)

*Allogeneic transplantation in patients with Low or Int-1 disease is generally considered at time of disease progression: progressive cytopenias and transfusion dependence, increasing blast counts, acquisition of adverse cytogenetic markers

**In view of the limited data on transplantation of adult patients with MDS using umbilical cord blood units, it is recommended that this should be performed within the confines of a clinical research protocol

International Prognostic Scoring System

	0	0.5	1	1.5	2
% BM blasts	<5	5-10		11-20	21-30
Cytopenias	0-1	2-3			
Karyotype*	Good	Intermediate	Poor		
Risk Category					
	Low risk	Int-1	Int-2	High risk	
Score	0	0.5-1	1.5-2	≥2.5	

*Good = normal, -Y, del(5q), del(20q)

Poor = complex(≥3 chromosome abnormalities) or chromosome 7 abnormalities

Intermediate = Changes not identified by the Good or Poor cytogenetic subgroups

References

1. Bowen, D., Culligan, D., Jowitt, S., Kelsey, S., Mufti, G., Oscier, D. & Parker, J. (2003). Guidelines for the diagnosis and therapy of adult myelodysplastic syndromes. *Br J Haematol*, 120, 187-200
2. Cutler, C.S., Lee, S.J., Greenberg, P., Deeg, H.J., Perez, W.S., Anasetti, C., Bolwell, B.J., Cairo, M.S., Gale, R.P., Klein, J.P., Lazarus, H.M., Liesveld, J.L., McCarthy, P.L., Milone, G.A., Rizzo, J.D., Schultz, K.R., Trigg, M.E., Keating, A., Weisdorf, D.J., Antin, J.H. & Horowitz, M.M. (2004). A decision analysis of allogeneic bone marrow transplantation for the myelodysplastic syndromes: delayed transplantation for low-risk myelodysplasia is associated with improved outcome. *Blood*, 104, 579-85
3. de Witte, T., Brand, R., van Biezen, A., Delforge, M., Biersack, H., Or, R., Meloni, G., Bandini, B., Sierra, J., Kroger, N., Gratwohl, A. & Niederwieser, D. (2006). The role of stem cell source in autologous hematopoietic stem cell transplantation for patients with myelodysplastic syndromes. *Haematologica*, 91, 750-6
4. Ho, A.Y., Pagliuca, A., Kenyon, M., Parker, J.E., Mijovic, A., Devereux, S. & Mufti, G.J. (2004). Reduced-intensity allogeneic haematopoietic stem cell transplantation for myelodysplastic syndrome and acute myeloid leukaemia with multilineage dysplasia using Fludarabine, Busulphan and Alemtuzumab (CAMPATH-1H)(FBC) conditioning. *Blood*
5. Martino, R., Iacobelli, S., Brand, R., Jansen, T., van Biezen, A., Finke, J., Bacigalupo, A., Beelen, D., Reiffers, J., Devergie, A., Alessandrino, E., Mufti, G.J., Barge, R., Sierra, J., Ruutu, T., Boogaerts, M., Falda, M., Jouet, J.P., Niederwieser, D. & de Witte, T. (2006). Retrospective comparison of reduced-intensity conditioning and conventional high-dose conditioning for allogeneic hematopoietic stem cell transplantation using HLA-identical sibling donors in myelodysplastic syndromes. *Blood*, 108, 836-46

6. Scott, B.L., Sandmaier, B.M., Storer, B., Maris, M.B., Sorrow, M.L., Maloney, D.G., Chauncey, T.R., Storb, R. & Deeg, H.J. (2006). Myeloablative vs nonmyeloablative allogeneic transplantation for patients with myelodysplastic syndrome or acute myelogenous leukemia with multilineage dysplasia: a retrospective analysis. *Leukemia*, 20, 128-35
7. Sorrow, M.L., Maris, M.B., Storb, R., Baron, F., Sandmaier, B.M., Maloney, D.G. & Storer, B. (2005). Hematopoietic cell transplantation (HCT) specific comorbidity index: a new tool for risk assessment before allogeneic HCT. *Blood*, 106, 2912-9
8. Tauro S, Craddock C, Peggs, K, Begum G, Mahendra P, Cook G, Marsh J, Milligan D, Goldstone A, Hunter A, Khwaja A, Chopra R, Littlewood T, Peniket A, Parker A, Russell N, Jackson G, Hale G, Mackinnon S Allogeneic stem cell transplantation using a reduced intensity conditioning (RIC) regimen has the capacity to produce durable remissions and long term disease free survival in patients with high risk acute myeloid leukemia (AML) and myelodysplasia (MDS) *J Clin Oncol* (2005) 23:9387-93

Section 8

Indications for Haematopoietic Stem Cell Transplant for Solid Tumours.

Ewing's sarcoma/PNET

	Autograft	Sibling transplant	MUD transplant
High risk disease as part of initial treatment plan	CO	GNR	GNR
CR>1	CO	GNR	BNR

Notes and references:

1. *Pediatr Blood Cancer* 2007;49(2):115-116. This showed benefit to conventional multimodality therapy for children with high-risk disease of conventional treatment
2. *Pediatr Blood Cancer* 2007;49(2):190-195. AL-Feris N et al. Does consolidation with autologous stem cell transplantation improve the outcome of children with metastatic or relapsed Ewing's sarcoma
3. *Med J Aust* 2009;190:121-5. Moore AS et al. Haematopoietic stem cell transplantation for children in Australia and New Zealand, 1998-2006: a report of the Australasian Bone Marrow and Transplant Recipient Registry and the Australian and New Zealand children's haematology oncology group
4. *Am J Clin Oncol* 2005;28(3):301-9. Laurence V et al. Long-term follow up of high dose chemotherapy with autologous stem cell rescue in adults with Ewing tumour
5. *J Clin Oncol* 2006;24(24):3997-4002. Oberlin O et al. Impact of high dose busulfan plus melphalan as consolidation in metastatic Ewing tumours: a study by the societe Francaise des Cancers de l'Enfant. This is the basis of Euro-Ewing trial
6. *J Cancer Res Clin Oncol* 2007;133:1-11. Engelhardt M et al. High dose chemotherapy and autologous peripheral stem cell transplantation in adult patients with high-risk or advanced Ewing and soft tissue sarcoma

Neuroblastoma

	Autograft	Sibling transplant	MUD transplant
Poor-risk disease	S	GNR	GNR
CR>1	CO	GNR	GNR

Notes and references

1. Now part of BACUP information as an option in advanced disease
2. Cancer Chemother Pharmacol 1986;16:165-169. Hartmann O et al. Treatment of advanced neuroblastoma with high dose chemotherapy and autologous bone marrow transplantation
3. Bone Marrow Transplantation 1997;20:543-551. Cohn SL et al. Treatment of poor-risk neuroblastoma patients with high-dose chemotherapy and autologous peripheral stem cell rescue

Germ Cell

	Autograft (including tandem procedure)	Sibling transplant	MUD transplant
CR>1	S	GNR	GNR
Refractory disease	CO	GNR	GNR

Notes and References:

1. Eur J Cancer 2008;44(2):237-241. Sammier C et al. Risk factors in germ cell tumour patients with relapse or progressive disease after first-line chemotherapy: evaluation of a prognostic score for survival after high dose chemotherapy
2. N Eng J Med 2007;357(4):340-348. Einhorn LH et al. High dose chemotherapy and stem cell rescue for metastatic germ cell tumours
3. Haematologica 2002;87:95-104. De Giorgi U et al. The status of high dose chemotherapy with haematopoietic stem cell transplantation in patients with germ cell tumour

Soft tissue Sarcoma

	Autograft	Sibling Transplant	MUD transplant
CR1	CO	GNR	GNR

Notes and References:

1. Bone Marrow Transplantation 2004;34:37-41. Kasper B et al. High-dose chemotherapy with autologous peripheral blood stem cell transplantation for bone and soft tissue
2. Oncology 2005;68:2-3. Kasper B et al. Is there an indication for high-dose chemotherapy in the treatment of bone and soft-tissue sarcoma

Breast

	Autograft	Sibling transplant	MUD transplant
Adjuvant	D	GNR	GNR
Metastatic	D	GNR	GNR

Ovary

	Autograft	Sibling transplant	MUD transplant
Any indication	D	GNR	GNR

Lung

	Autograft	Sibling transplant	MUD transplant
Any indication	D	GNR	GNR

Renal

	Autograft	Sibling transplant	MUD transplant
Any indication	GNR	D	D

Section 9:

Myelofibrosis

	Sibling Transplant	MUD Transplant	Reduced Intensity Allo/MUD	Autograft
Primary Myelofibrosis (for prognostic score see ¹) • Low Risk • Intermediate Risk • High Risk	GNR CO (<45 yrs) S (<45 yrs)	GNR CO (<45 yrs) S (<45 yrs)	GNR CO (>45 yrs) S (>45 yrs) CO (<45 if clinically unfit)	GNR CO ² CO ²
Secondary Myelfibrosis • Post-PV MF • Post-ET MF	CO CO	CO CO	CO CO	GNR GNR

¹Dupriez B, Morel P, Demory J-L et al. (1996) Prognostic factors in agnogenic myeloid metaplasia: a report on 195 cases with a new scoring system. Blood 88, 1013-1018.

² Best results if 'rainy day' harvest obtained at diagnosis

The Lille Scoring System

No of adverse factors	Risk Group	Cases (%)	Median Survival
0	low	47	93
1	intermediate	45	26
2	high	8	13

Myeloablative regimen

- Deeg HJ, Gooley TA, Flowers ME et al, (2003). Allogeneic hematopoietic stem cell transplantation for myelofibrosis. *Blood*, 102, 3912-3918.
- Guardiola P, Andersen JE, Bandini G et al. Allogeneic stem cell transplantation for agnogenic myeloid metaplasia, *Blood* 1999, 93, 2831-2838.
- Guardiola P, Andersen JE, Gluckman E. Myelofibrosis with myeloid metaplasia. (2000). *N Eng J Med*, 343, 659-660.

Reduced intensity conditioning regimen

- Rondelli D, Barosi G, Bacigalupo A et al (2005) Allogeneic hematopoietic stem-cell transplantation with reduced-intensity conditioning in intermediate-or high risk patients with myelofibrosis with myeloid metaplasia. *Blood*, 105, 4115-4119.
- Merup M, Lazarevic V, Nahi H et al (2006) Different outcome of allogeneic transplantation in myelofibrosis using conventional or reduced- intensity conditioning regimens. *Br J Haematol*, 135, 367-373.

Section 10

Indications for Haematopoietic Stem Cell Transplant for Severe Autoimmune Diseases

Based on Ljungman et al. Allogeneic and autologous transplantation for haematological diseases, solid tumours and immune disorders: definitions and current practice in Europe 2008. Bone Marrow Transplantation 2009: submitted)

*All patients with the specified diseases must be deemed to have severe treatment resistant disease and sufficiently fit for transplant procedure as determined by appropriate multi-specialty review.

Autologous HSCT for other autoimmune disorders is considered as developmental.

Multiple sclerosis

	Sibling transplant	MUD transplant	Autograft
Severe, resistant disease*	GNR	GNR	CO

References:

1. Saccardi, R., Kozak, T., Bocelli-Tyndall, C., Fassas, A., Kazis, A., Havrdova, E., Carreras, E., Saiz, A., Lowenberg, B., te Boekhorst, P.A., Gualandio, F., Openshaw, H., Longo, G., Pagliai, F., Massacesi, L., Deconink, E., Ouyang, J., Nagore, F.J., Besalduch, J., Lisukov, I.A., Bonini, A., Merelli, E., Slavino, S., Gratwohl, A., Passweg, J., Tyndall, A., Steck, A.J., Andolina, M., Capobianco, M., Martin, J.L., Lugaresi, A., Meucci, G., Saez, R.A., Clark, R.E., Fernandez, M.N., Fouillard, L., Herstenstein, B., Koza, V., Cocco, E., Baurmann, H. & Mancardi, G.L. (2006) Autologous stem cell transplantation for progressive multiple sclerosis: update of the European Group for Blood and Marrow Transplantation autoimmune diseases working party database. *Mult Scler*, 12, 814-823
2. Burt RK , [Loh Y](#), [Cohen B](#), [Stefoski D](#), [Balabanov R](#), [Katsamakidis G](#), [Oyama Y](#), [Russell EJ](#), [Stern J](#), [Muraro P](#), [Rose J](#), [Testori A](#), [Bucha J](#), [Jovanovic B](#), [Milanetti F](#), [Storek J](#), [Voltarelli JC](#), [Burns WH](#) Autologous non-myeloablative haemopoietic stem cell transplantation in relapsing-remitting multiple sclerosis: a phase I/II study. *Lancet Neurol*. 2009 Mar;8(3):244-53.
3. Roccatagliata, L., Rocca, M., Valsasina, P., Bonzano, L., Sormani, M., Saccardi, R., Mancardi, G. & Filippi, M. (2007) The long-term effect of AHSCT on MRI measures of MS evolution: a five-year follow-up study. *Mult Scler*, 13, 1068-1070
4. Portaccio, E., Amato, M.P., Siracusa, G., Pagliai, F., Sorbi, S., Guidi, S., Bosi, A. & Saccardi, R. (2007) Autologous hematopoietic stem cell transplantation for very active relapsing-remitting multiple sclerosis: report of two cases. *Mult Scler*, 13, 676-678

Systemic sclerosis

	Sibling transplant	MUD transplant	Autograft
Severe, resistant disease*	D	GNR	CO

References:

1. Vonk, M.C., Marjanovic, Z., van den Hoogen, F.H., Zohar, S., Schattenberg, A.V., Fibbe, W.E., Larghero, J., Gluckman, E., Preijers, F.W., van Dijk, A.P., Bax, J.J., Roblot, P., van Riel, P.L., van Laar, J.M. & Farge, D. (2008) Long-term follow-up results after autologous haematopoietic stem cell transplantation for severe systemic sclerosis. *Ann Rheum Dis*, 67, 98-104
2. Farge, D., Passweg, J., van Laar, J.M., Marjanovic, Z., Besenthal, C., Finke, J., Peter, H.H., Breedveld, F.C., Fibbe, W.E., Black, C., Denton, C., Koetter, I., Locatelli, F., Martini, A., Schattenberg, A.V., van den Hoogen, F., van de Putte, L., Lanza, F., Arnold, R., Bacon, P.A., Bingham, S., Ciceri, F., Didier, B., Diez-Martin, J.L., Emery, P., Feremans, W., Hertenstein, B., Hiepe, F., Luosujarvi, R., Leon Lara, A., Marmont, A., Martinez, A.M., Pascual Cascon, H., Bocelli-Tyndall, C., Gluckman, E., Gratwohl, A. & Tyndall, A. (2004) Autologous stem cell transplantation in the treatment of systemic sclerosis: report from the EBMT/EULAR Registry. *Ann Rheum Dis*, 63, 974-981.
3. Nash, R.A., McSweeney, P.A., Crofford, L.J., Abidi, M., Chen, C.S., Godwin, J.D., Gooley, T.A., Holmberg, L., Henstorf, G., LeMaistre, C.F., Mayes, M.D., McDonagh, K.T., McLaughlin, B., Molitor, J.A., Nelson, J.L., Shulman, H., Storb, R., Viganego, F., Wener, M.H., Seibold, J.R., Sullivan, K.M. & Furst, D.E. (2007) High-dose immunosuppressive therapy and autologous hematopoietic cell transplantation for severe systemic sclerosis: long-term follow-up of the US multicenter pilot study. *Blood*, 110, 1388-1396
4. Oyama, Y., Barr, W.G., Statkute, L., Corbridge, T., Gonda, E.A., Jovanovic, B., Testori, A. & Burt, R.K. (2007) Autologous non-myeloablative hematopoietic stem cell transplantation in patients with systemic sclerosis. *Bone Marrow Transplant*, 40, 549-555
5. Nash, R.A., McSweeney, P.A., Nelson, J.L., Wener, M., Georges, G.E., Langston, A.A., Shulman, H., Sullivan, K.M., Lee, J., Henstorf, G., Storb, R. & Furst, D.E. (2006) Allogeneic marrow transplantation in patients with severe systemic sclerosis: resolution of dermal fibrosis. *Arthritis Rheum*, 54, 1982-1986
6. Khorshid, O., Hosing, C., Bibawi, S., Ueno, N., Reveille, J., Mayes, M.D. & Champlin, R.E. (2004) Nonmyeloablative stem cell transplant in a patient with advanced systemic sclerosis and systemic lupus erythematosus. *J Rheumatol*, 31, 2513-2516
7. Loh, Y., Oyama, Y., Statkute, L., Verda, L., Quigley, K., Yaung, K., Barr, W., Jovanovic, B. & Burt, R.K. (2007) Non-myeloablative allogeneic hematopoietic stem cell transplantation for severe systemic sclerosis: graft-versus-autoimmunity without graft-versus-host disease? *Bone Marrow Transplant*, 39, 435-437

Systemic lupus erythematosus

	Sibling transplant	MUD transplant	Autograft
Severe, resistant disease*	D	GNR	CO

References:

1. Burt, R.K., Traynor, A., Statkute, L., Barr, W.G., Rosa, R., Schroeder, J., Verda, L., Krosnjar, N., Quigley, K., Young, K., Villa Bs, M., Takahashi, M., Jovanovic, B. & Oyama, Y. (2006b) Nonmyeloablative hematopoietic stem cell transplantation for systemic lupus erythematosus. *Jama*, 295, 527-535
2. Jayne, D., Passweg, J., Marmont, A., Farge, D., Zhao, X., Arnold, R., Hiepe, F., Lisukov, I., Musso, M., Ou-Yang, J., Marsh, J., Wulfraat, N., Besalduch, J., Bingham, S.J., Emery, P., Brune, M., Fassas, A., Faulkner, L., Ferster, A., Fiehn, C., Fouillard, L., Geromin, A., Greinix, H., Rabusin, M., Saccardi, R., Schneider, P., Zintl, F., Gratwohl, A. & Tyndall, A. (2004) Autologous stem cell transplantation for systemic lupus erythematosus. *Lupus*, 13, 168-176
3. Lu, Q., Lu, L., Niu, X., Guo, Y., Parino, G.R. & Liu, D. (2006) Non-myeloablative allogeneic stem cell transplant in a patient with refractory systemic lupus erythematosus. *Bone Marrow Transplant*, 37, 979-981
4. Khorshid, O., Hosing, C., Bibawi, S., Ueno, N., Reveille, J., Mayes, M.D. & Champlin, R.E. (2004) Nonmyeloablative stem cell transplant in a patient with advanced systemic sclerosis and systemic lupus erythematosus. *J Rheumatol*, 31, 2513-2516

Rheumatoid arthritis

	Sibling transplant	MUD transplant	Autograft
Severe, resistant disease*	GNR	GNR	CO

References:

1. Snowden, J.A., Kapoor, S. & Wilson, A.G. (2008) Stem cell transplantation in rheumatoid arthritis. *Autoimmunity*, 2008 Oct 28:1. [Epub ahead of print]
2. Snowden, J.A., Passweg, J., Moore, J.J., Milliken, S., Cannell, P., Van Laar, J., Verburg, R., Szer, J., Taylor, K., Joske, D., Rule, S., Bingham, S.J., Emery, P., Burt, R.K., Lowenthal, R.M., Durez, P., McKendry, R.J., Pavletic, S.Z., Espigado, I., Jantunen, E., Kashyap, A., Rabusin, M., Brooks, P., Bredeson, C. & Tyndall, A. (2004) Autologous hemopoietic stem cell transplantation in severe rheumatoid arthritis: a report from the EBMT and ABMTR. *J Rheumatol*, 31, 482-488.

Crohn's disease

	Sibling transplant	MUD transplant	Autograft
Severe, resistant disease*	GNR	GNR	CO

References:

1. Oyama, Y., Craig, R.M., Traynor, A.E., Quigley, K., Statkute, L., Halverson, A., Brush, M., Verda, L., Kowalska, B., Krosnjar, N., Kletzel, M., Whittington, P.F. & Burt, R.K. (2005) Autologous hematopoietic stem cell transplantation in patients with refractory Crohn's disease. *Gastroenterology*, 128, 552-563
2. Cassinotti A, Annaloro C, Ardizzone S, Onida F, Della Volpe A, Clerici M, Usardi P, Greco S, Maconi G, Porro GB, Deliliers GL. Autologous haematopoietic stem cell transplantation without CD34+ cell selection in refractory Crohn's disease. *Gut*. 2008 Feb;57(2):211-7

Immune cytopenias

	Sibling transplant	MUD transplant	Autograft
Severe, resistant ITP, AIHA, Evans syndrome*	D	D	D

References:

1. Passweg, J.R., Rabusin, M., Musso, M., Beguin, Y., Cesaro, S., Ehninger, G., Espigado, I., Iriundo, A., Jost, L., Koza, V., Lenhoff, S., Lisukov, I., Locatelli, F., Marmont, A., Philippe, P., Pilatino, C., Quartier, P., Stary, J., Veys, P., Vormoor, J., Wahlin, A., Zintl, F., Bocelli-Tyndall, C., Tyndall, A. & Gratwohl, A. (2004) Haematopoietic stem cell transplantation for refractory autoimmune cytopenia. *Br J Haematol*, 125, 749-755
2. Urban, C., Lackner, H., Sovinz, P., Benesch, M., Schwinger, W., Dornbusch, H.J. & Moser, A. (2006) Successful unrelated cord blood transplantation in a 7-year-old boy with Evans syndrome refractory to immunosuppression and double autologous stem cell transplantation. *Eur J Haematol*, 76, 526-530

Chronic inflammatory demyelinating polyneuropathy (CIDP)

	Autograft	Sibling transplant	MUD transplant
Severe, resistant disease*	D	GNR	GNR

References:

1. Oyama Y, Sufit R, Loh Y, Statkute L, Yaung K, Quigley K, Gonda E, Spahovic D, Bronesky D, Burt RK. Nonmyeloablative autologous hematopoietic stem cell transplantation for refractory CIDP. *Neurology*. 2007 Oct 30;69(18):1802-3
2. Vermeulen, M. & Van Oers, M.H. (2002) Successful autologous stem cell transplantation in a patient with chronic inflammatory demyelinating polyneuropathy. *J Neurol Neurosurg Psychiatry*, 72, 127-128
3. Mahdi-Rogers M., Kazmi M., Ferner R., Hughes R.A.C., Renaud S., Steck A., Fuhr P., Halter J., Gratwohl A., Tyndall A. Autologous peripheral blood stem cell transplantation for chronic acquired demyelinating neuropathy *Journal of the Peripheral Nervous System* 2009 in press.