

Appendix 1 (as supplied by the authors): Methods and results of studies of autoHCT for SSc*

Study	N	Patient characteristics	HSC mobilization	Conditioning (or control Rx)	CD34 selection	Med F/U (y)	TRM	Efficacy
Non-randomized								
Binks M: Ann Rheum Dis 2001, 60:577 ¹	41	Age 41 (med) Dis.dur. ~2 y mRSS 29 FVC <70% in ½ pts	Cy 4 g/m ² + GCSF (most pts)	Cy 150-200 mg/kg (most pts)	Yes (most pts)	1	17%	OS at 1 y 73% mRSS improved Lung function stable
Farge D: Brit J Haematol 2002, 119:726 ²	11	Age 46 (med) Dis.dur. ~2 y mRSS 29 FVC 67%	Cy 4 g/m ² + GCSF	Cy 200 mg/kg (most pts)	Yes	1 ½	9%	OS at 1 ½ y 64% mRSS improved QOL improved
Nash RA: Blood 2007, 110:1388 ³	34	Age 41 (med) Dis.dur. <4 y mRSS 30 FVC 72%	GCSF	Cy 120 mg/kg + TBI 8 Gy + Atgam 90 mg/kg	Yes	5	24%	OS at 5 y 64% PFS at 5 y 64% mRSS improved Lung function stable QOL improved
Oyama Y: Bone Marrow Transplant 2007, 40:549 ⁴	10	Age 46 (med) Dis.dur. ~3 y mRSS 30 FVC ~70%	Cy 2 g/m ² + GCSF	Cy 200 mg/kg + Thymoglob. 7.5 mg/kg	No	2	0%	OS at 2 y 90% PFS at 2 y 70% mRSS improved Lung function stable
Vonk MC: Ann Rheum Dis 2008, 67:98 ⁵	26	Age 42 (med) Dis.dur. ~2 y mRSS 32 FVC 76%	Cy 4 g/m ² + GCSF	Cy 200 mg/kg	Yes	5	4%	OS at 5 y 96% PFS at r y 64% mRSS improved Lung function stable
Tsukamoto H: Rheumatol 2011, 50:944 ⁶	11	Age 52 (avg) Dis.dur. <5 y mRSS 22 FVC 65%	Cy 4 g/m ² + GCSF	Cy 200 mg/kg	Yes	5	0%	OS at 3 y 91% mRSS improved FVC 65→78% DLCO stable ↓ Scl70, TNF, TGF
Randomized								
Burt: Lancet 2011, 378:498 ⁷ (ASSIST)	10 vs 9 controls	Age 45 (med) Disease duration ~1 y Cy <6 IV doses mRSS ~23 FVC ~65%	Cy 2 g/m ² + GCSF	Cy 200 mg/kg + Thymoglob. 6.5 mg/kg (w M-pred 1 g x 4) vs Cy 1 g/m ² monthly x 6	No	1	0% vs 0%	OS @ 1 y 100% vs 100% Evaluations at BL and at 1 y: mRSS 28→15 vs 19→22 FVC 62→74% vs 67→61% QOL (SF36 total score) 39→56% vs 50→40% (all differences between groups significant, except for OS)
Van Laar: J Amer Med Association 2014, 311:2490 ⁸ (ASTIS)	79 vs 77 controls **	Age 44 (avg) Disease duration ~1 y Cy <5 g IV total mRSS 25 (avg) FVC 81% (avg)	Cy 4 g/m ² + GCSF	Cy 200 mg/kg + Thymoglob. 7.5 mg/kg (w M-pred 1 mg/kg x3) vs Cy 750 mg/m ² monthly x 12	Yes	6**	10% vs 0%	OS @ 4 y 86% vs 76% EFS @ 4 y 81% vs 74% (event = death or irreversible organ failure) Changes from BL to 2 y: mRSS decrease, 20 vs 9 FVC increase, 5 vs -1% QOL (SF36 physical score) Increase, 10 vs 4 (all significant)
Sullivan: Arthritis Rheumatol 2016, 68 (Suppl 10):	36 vs 39 controls	Age 18-69 Disease Duration ≤5 y Cy up to 4-6 mo mRSS 30 (avg)	GCSF	Cy 120 mg/kg + TBI 8 Gy + Atgam 90 mg/kg vs	Yes	>5	3% vs 0%	OS @ 4 ½ y 91% vs 77% EFS @ 4 ½ y 79% vs 50% (event = death or renal/cardiac/pulmonary failure)

Appendix to: Storek J, Daly A, LeClercq SA. Autologous hematopoietic cell transplantation for systemic sclerosis — a challenge for the Canadian health care system. *CMAJ* 2017. doi:10.1503/cmaj.161346. Copyright © 2017 The Author(s) or their employer(s).

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Abstract 6L ⁹ (SCOT)		FVC 74% (avg)		Cy 750 mg/m ² monthly x12				(all significant)
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* Only studies with ≥10 patients are shown.

** Only 71 vs 57 patients completed treatment, and 8 controls received HCT at ≥2 y. The analyses under Efficacy are intention-to-treat analyses.

Abbreviations: Dis.dur., disease duration; med, median; mRSS, modified Rodnan skin score; FVC, forced vital capacity; Cy, cyclophosphamide; GCSF, granulocyte colony stimulating factor (filgrastim); HCT, hematopoietic cell transplantation; F/U, follow up; TRM, transplant related mortality; TBI, total body irradiation; Thymoglob., Thymoglobulin; M-pred, methylprednisolone; pts, patients; OS, overall survival; PFS, progression-free survival; EFS, event-free survival; QOL, quality of life; TNF , tumor necrosis factor alpha; TGF , transforming growth factor beta; BL, baseline;

References of Appendix 1

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