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Recommendation Report SCT-2 Version 2

Stem Cell Transplantation in Primary Systemic Amyloidosis

Members of the Stem Cell Transplantation Expert Panel

An assessment conducted in February 2024 deferred the review of Recommendation Report SCT-2 Version 2. This means that the document remains current until it is assessed again next year. The PEBC has a formal and standardized process to ensure the currency of each document (PEBC Assessment & Review Protocol)

Recommendation Report SCT-2 Version 2 is comprised of 3 sections. You can access the summary and full report here: https://www.cancercareontario.ca/en/guidelines-advice/types-of-cancer/981

Section 1: Recommendations (ENDORSED)
Section 2: Summary of Methods and Evidence
Section 3: Document Assessment and Review

November 18, 2019

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Recommendation Report Citation (Vancouver Style): Kouroukis CT, Rumble RB. Stem cell transplantation in primary systemic amyloidosis. Bhella S, Varela N, reviewers. Toronto (ON): Cancer Care Ontario (CCO); 2012 Mar 29 [Endorsed 2019 Nov]. Program in Evidence-based Care (PEBC) Recommendation Report No.: SCT-2 Version 2 ENDORSED.

Recommendation Report History

GUIDELINE VERSION	SYSTEMATIC REVIEW		PUBLICATIONS	NOTES and KEY CHANGES
	Search Dates	Data		
Original March 29, 2012	2006 to Oct 2010	Full Report	Web publication	N.A.
Version 2 November 18, 2019	2010 to Jun 2019	New data found in Section 3: Document Assessment and Review	Updated web publication	2012 recommendations are ENDORSED

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Recommendation Report SCT-2 Version 2: Section 1

Stem Cell Transplantation in Primary Systemic Amyloidosis: Recommendations

The 2012 recommendations have been ENDORSED. This means that the recommendations are still current and relevant for decision making. Please see Section 3: Document Assessment and Review for a summary of updated evidence published between 2010 and 2019, and for details on how this Recommendation Report was ENDORSED.

CLINICAL QUESTION

What is the role of stem cell transplantation (SCT) in the treatment of primary systemic (AL, amyloid light-chain) amyloidosis?

TARGET POPULATION

All adult patients with primary (AL) amyloidosis who are being considered for treatment that includes either bone marrow or SCT.

RECOMMENDATIONS AND KEY EVIDENCE

High-dose chemotherapy (CT) and autologous SCT is an option for selected patients with primary systemic amyloidosis, preferably within an investigative setting.

Evidence

A single meta-analysis (1) met the inclusion criteria for this review, and that meta-analysis found no significant difference between autologous stem cell transplantation (ASCT) and CT for AL patients in survival outcomes. An RCT included in that meta-analysis (Jaccard et al, 2007) found treatment with ASCT to be associated with a significant increase in treatment-related mortality (TRM).

This meta-analysis has some limitations that must be considered when making evidence-based recommendations. The quality of the included evidence was low and consisted of a small RCT and non-RCTs with likely patient selection bias. The single included RCT needed 340 patients to detect a 15% survival difference at $\propto$=0.05, but only 100 were accrued. Secondly, AL patients typically also have significant co-morbidities precluding them from study enrolment.

In consideration of the lack of curative treatment options and the limitations of the evidence reviewed, the Expert Panel believes that offering ASCT may be a reasonable option for some patients, depending on performance status, co-morbidities, patient preferences, and ultimate treatment goals.

Allogeneic SCT is not recommended for patients with primary systemic amyloidosis.

Evidence
There is no evidence supporting the use of allogeneic SCT for patients with AL.

QUALIFYING STATEMENT

The patient selection process and the ultimate decision to perform an SCT should take into account not only disease-related characteristics, but also comorbidities and patient preferences.

Added to the 2019 Endorsement:

Careful patient selection based on degree of light chain amyloidosis involvement and organ function is an emerging concept in amyloidosis that should be considered to reduce transplant-related mortality.

Transplantation in amyloidosis is an evolving area. New emerging areas include consideration of transplantation in first relapse and the impact of novel proteasome inhibitors on outcomes. New evidence is expected within a time frame of 2 to 3 years.

FUTURE RESEARCH

Newer agents are being investigated in the treatment of AL amyloidosis. At this time it is not known how they may impact the need for SCT.

IMPLICATIONS FOR POLICY

The number of transplants provincially for systemic AL amyloidosis remains very low, and is unlikely to change in the foreseeable future.

RELATED PROGRAM IN EVIDENCE-BASED CARE REPORTS

- Imrie K, Rumble RB, Crump M; Advisory Panel on Bone Marrow and Stem Cell Transplantation; Hematology Disease Site Group of Cancer Care Ontario's Program in Evidence-based Care. Stem cell transplantation in adults. [Report Date: January 30, 2009] (2). Available from: http://www.cancercare.on.ca/common/pages/UserFile.aspx?fileId=35448

Funding

The PEBC is a provincial initiative of Cancer Care Ontario supported by the Ontario Ministry of Health and Long-Term Care through Cancer Care Ontario. All work produced by the PEBC is editorially independent from its funding source.

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1. Mhaskar R, Kumar A, Behera M, Kharfan-Dabaja MA, Djulbegovic B. Role of high-dose chemotherapy and autologous hematopoietic cell transplantation in primary systemic amyloidosis: a systematic review. Biol Blood Marrow Transplant. 2009;15(8):893-902.
2. Imrie K, Rumble RB, Crump M. Stem cell transplantation in adults. Toronto: Cancer Care Ontario; 2009 [cited 2011 March 28, 2011]; Available from: http://www.cancercare.on.ca/common/pages/UserFile.aspx?fileId=35448.