RHEUMATOLOGY

Rheumatology 2020;59:2052–2061
doi:10.1093/rheumatology/kez579
Advance Access publication 6 December 2019

Original article

Treatment decision-making in diffuse cutaneous systemic sclerosis: a patient's perspective

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Abstract

Objectives. To examine the treatment decision-making process of patients with dcSSc in the context of haematopoietic stem cell transplantation (HSCT).

Methods. A qualitative semi-structured interview study was done in patients before or after HSCT, or patients who chose another treatment than HSCT. Thematic analysis was used. Shared decision-making (SDM) was assessed with the 9-item Shared Decision Making Questionnaire (SDM-Q-9).

Results. Twenty-five patients [16 male/nine female, median age 47 (range 27–68) years] were interviewed: five pre-HSCT, 16 post-HSCT and four following other treatment. Whereas the SDM-Q-9 showed the decision-making process was perceived as shared [median score 81/100 (range 49–100)], we learned from the interviews that the decision was predominantly made by the rheumatologist, and patients were often steered towards a treatment option. Strong guidance of the rheumatologist was appreciated because of a lack of accessible, reliable and SSc-specific information, due to the approach of the decision-making process of the rheumatologist, the large consequence of the decision and the trust in their doctor. Expectations of outcomes and risks also differed between patients. Furthermore, more than half of patients felt they had no choice but to go for HSCT, due to rapid deterioration of health and the perception of HSCT as 'the holy grail'.

Conclusion. This is the first study that provides insight into the decision-making process in dcSSc. This process is negatively impacted by a lack of disease-specific education about treatment options. Additionally, we recommend exploring patients' preferences and understanding of the illness to optimally guide decision-making and to provide tailor-made information.

Key words: SSc, haematopoietic stem cell transplantation, shared decision-making, decision-making, qualitative research, patient perspective

Rheumatology key messages

- Decision-making is strongly guided by the rheumatologist in patients with diffuse cutaneous systemic sclerosis.
- Decision-making in dcSSc is influenced by health status, prospects, knowledge, expectations, patient–physician and social interaction.
- The lack of disease-specific and accessible information hinders dcSSc patients in making a balanced decision.

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Submitted 28 July 2019; accepted 22 October 2019

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Introduction

SSc is a debilitating and incurable autoimmune connective tissue disease. The 5-year mortality for dcSSc with rapid increase in skin involvement and development of organ fibrosis is ∼25% [1, 2]. In dcSSc, immunosuppressive or cytotoxic agents such as mycophenolate mofetil, methotrexate and cyclophosphamide are widely used [3]. Autologous haematopoietic stem cell transplantation (HSCT) is often regarded as the last treatment option in dcSSc. HSCT has been shown to lead to superior outcomes with regard to survival, quality of life (QoL), skin fibrosis and prevention of disease progression in comparison with intravenous cyclophosphamide [4–6]. HSCT has since been implemented in (inter)national treatment guidelines for dcSSc and is offered in clinical care [7, 8]. There are, however, no specific guidelines on patient selection or optimal timing for HSCT, and the value of HSCT as treatment regimen compared with other available immunosuppressive therapies is unclear. Importantly, despite evidence for the superior long-term benefits, HSCT carries a treatment-related mortality between 3% and 10% in the first year following treatment [9]. Therefore, treatment choice is primarily based on the preferences of the patient and rheumatologist. It is important that rheumatologists and multidisciplinary teams treating patients with dcSSc facilitate the patient's arrival at a decision that is aligned to his or her preferences.

Shared decision-making (SDM) is defined as 'an approach where clinicians and patients share the best available evidence when faced with the task of making decisions, and where patients are supported to consider options, to achieve informed preferences' [10]. SDM is a means to incorporate patient preferences in treatment decisions [11]. Unfortunately, there is not much experience with regard to SDM and optimal patient education for HSCT in dcSSc or other life-threatening autoimmune diseases [12]. The aim of this study was to get insight in and improve understanding of the decision-making process in dcSSc in order to identify ways to support SDM in this group of patients.

Methods

Design

In this exploratory qualitative study, interviews were conducted and thematically analysed [13–15].

Patients

A purposeful sample of patients with dcSSc who were scheduled for HSCT, who had already undergone HSCT, or who chose another treatment than HSCT were recruited from the four university hospitals in the Netherlands that offer HSCT in dcSSc (University Medical Centre Utrecht, Leiden University Medical Centre, Amsterdam UMC, Vrije Universiteit, Radboud University Medical Centre Nijmegen). Heterogeneity was sought with regard to treatment history, outcomes, participation in a randomized clinical trial, disease duration, marital status, level of education, age and gender. Non-Dutch speaking patients were excluded. Rheumatologists were informed about the sampling criteria, and approached the patients. Consenting patients were invited for an interview at a convenient moment and written informed consent was obtained prior to the interview. This study was classified by the institutional review board as exempt from the Medical Research Involving Human Subjects Act (17-836/C). This study was conducted with the approval of the institutional review boards and ethics committees of Utrecht, Leiden, Amsterdam and Nijmegen (University Medical Centres, The Netherlands).

Data collection

To facilitate the in-depth, semi-structured, face-to-face interviews, an interview guide with open-ended questions and a topic list was made (see Supplementary section Interview guide, available at *Rheumatology* online) [16]. Questions aimed at exploring the decision-making process, including information provision, patient expectations, patient–physician interaction and external factors. On the day of the interview, prior to the interview, patients completed a questionnaire assessing sociodemographic and disease characteristics. The extent to which patients perceived that SDM had occurred was assessed using the 9-item Shared Decision Making Questionnaire (SDM-Q-9), which contains nine items scored on a six-point Likert scale from 0 (completely disagree) to 5 (completely agree). Total transformed scores range from 0 to 100 [17]; higher scores denote higher perceived SDM [see Supplementary section Shared-decision making Questionnaire 9 (SDM-Q-9), available at *Rheumatology* online]. Daily functioning and health-related QoL were assessed using the Scleroderma Health Assessment Questionnaire (range scores: 0–3) [18] and the EuroQoL 5 Dimension 5 Levels (range scores: 0–1) [19], respectively. Higher scores on the scales denote worse functioning and better health-related QoL, respectively (see Supplementary sections Scleroderma Health assessment questionnaire and EurolQoL-5 dimensions-5 levels, Health related quality of life questionnaire, available at *Rheumatology* online). The SDM-Q, EuroQoL 5 Dimension 5 Levels and Scleroderma Health Assessment Questionnaire questionnaires were completed on the same day as the interview. The interview was conducted by one investigator (J.S. or F.R.). First, a senior researcher (M.K.) read and discussed the transcripts of the first two interviews in order to improve the interview style and technique to collect rich data. Interview experiences were shared to foster similarity in how the patients were approached, i.e. the used interviewing techniques and setting of the interview. Prior to the interview, the investigator explained the research goals, and took time to answer questions and make patients feel at ease [20]. At inclusion names of patients were replaced by a code. An

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independent researcher (J.dB.) transcribed the interviews verbatim and anonymized the transcripts (e.g. names of persons and hospitals were deleted from the texts) [21]. The manuscript was read and evaluated by a representative of the patient sounding board (P.M.).

Data analyses

An inductive thematic analysis was performed, themes were identified and described [14, 22–25]. Transcripts were analysed using the constant comparative technique [14]. The two interviewers independently coded the data, using the software programme NVivo12 [26]. Codes are meaningful fragments in relation to the research, and the first step was to sort the data for further interpretation [14]. Contradictions were discussed aimed to reach intersubjective agreement. The different codes were grouped into themes and subthemes. Themes are defined as patterns of meaning resulting from the codes; subthemes are patterns identified within a certain theme. The categorization, definition and refining of themes and subthemes was done by J.S. and independently checked by F.R., J.dB. and M.K.

Saturation was assessed at a conceptual level [25]. F.R. and J.S. determined if a new interview added new codes. When data saturation was reached, inclusion of patients was closed. The consolidated criteria for reporting qualitative research (COREQ) were followed and reported in the Supplementary section COREQ checklist (COnsolidated criteria for REporting Qualitative research) available at *Rheumatology* online) [27]. Scores of the questionnaires were calculated according to validated formulas. Quantitative data were analysed using SPSS Statistics version 21.0 (IBM Corp., Armonk, NY, USA). Descriptive statistics (median and range) were used to present socio-economic data and disease characteristics.

Results

Patients

Twenty-six patients were invited to participate in the study. One patient declined participation due to poor health condition. Twenty-five patients were interviewed. Patient characteristics are shown in Table 1. Patients were interviewed face-to-face at the hospital after a scheduled medical appointment ($n = 23$), at their office ($n = 1$), or at their home ($n = 1$).

Treatment decision-making approaches

Three different approaches to decision-making were identified from the patients' stories. Six patients reported that options were presented sequentially, with the next option offered only when the previous option had failed or the patient had declined it (Fig. 1A). Nine patients were offered multiple treatment options as part of a stepwise treatment plan, with an *a priori* preferred order of options (Fig. 1B). Ten patients were offered multiple options without an *a priori* order, which was the preferred approach according to patients (Fig. 1C). Four patients who were offered treatment options sequentially mentioned that they could not see all treatment options in perspective or get a clear overview of alternative treatments. They felt they did not really have a choice.

Across the centres and across rheumatologists, there were differences in approaches, but these differences were not significant due to the small sample.

Identified themes in the decision-making process

Six main themes were identified to play a role in patients' decision-making. The themes with subthemes and associated issues are shown in Table 2. Illustrative quotes are presented in Table 3.

Poor prospects and low QoL: leaving no other option

Prior to the decision-making process, most patients ($n = 22$) were shocked to learn that dcSSc has a poor prognosis, especially as none of the patients except one had ever heard of SSc before. Some patients associated the information they received about survival and therapeutic options (i.e. chemotherapy and HSCT) with cancer. They sometimes felt that people in their social networks did not fully understand the burden and severity of their condition; because SSc is unknown, their symptoms are not visible, and other rheumatic diseases are usually associated with less severe and treatable symptoms.

QoL and health status were important aspects in decision-making. Patients reported that dcSSc had either a very large negative impact on their QoL and daily functioning, or their general health was rapidly deteriorating at the time the decision had to be made. Five patients believed that a condition with such a fast-progressing course necessitated high intensity therapy such as HSCT at short notice. Some patients mentioned that they accepted the potential risk of treatment complications, even fatal complications, considering their low QoL. Some patients had experienced failure of other therapies and therefore thought HSCT was the best, if not the only, option left to commence. Patients who opted for other therapies than HSCT felt they had the time to try alternatives and leave HSCT as a last rescue option.

Expectations: maximizing chances for survival?

Expectations on the outcomes of HSCT varied among patients. Most patients mentioned that they had expected that HSCT provided the best, if not the only, chance for survival. HSCT was regarded by many patients as the ultimate treatment, as 'the holy grail'.

Some expected that HSCT could completely cure them. Patients hoped to get back to their pre-diagnosis activity level. Return to work was an important consideration in the decision-making process. Most patients had to discontinue working due to ill health, which led to loss of financial independence and identity. They anticipated that HSCT was the only way to return to work.

One patient thought the duration and impact of the treatment regimen in HSCT was too intense to combine

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Table 1 Patient characteristics

Characteristic	Total ($n = 25$)	Prior to HSCT ($n = 5$)	Post-HSCT ($n = 16$)	Other treatment ($n = 4$)
Age, median (range), years	47 (27–68)	41.0 (36–57)	47 (27–68)	45 (43–48)
Male sex, *n*	16	5	9	2
Marital status, *n*				
Married	21	4	13	4
Living together unmarried	2	0	2	0
Unmarried	2	1	1	0
Household, *n*				
Living alone	1	1	0	0
Living with parents	1	0	1	0
Living with partner	7	0	7	0
Living with partner and children	16	4	8	4
Educational level, *n*				
Low (primary and secondary school)	6	0	5	1
Medium (high school)	11	4	5	2
High (graduate and above)	8	1	6	1
Participation in a randomized clinical trial, *n*	2	0	2	0
Paid job at time of interview, *n*	18	3	12	3
Disease duration, median (range), years	4.3 (0.2–12.0)	1.0 (0.5–1.0)	4.0 (2.0–13.0)	4.0 (2.5–6.0)
Disease duration at decision, median (range), years	1.4 (0.1–6.0)	1.0 (0.5–1.0)	0.7 (0.1–2.0)	1.0 (1.0–4.0)
Time between decision and interview, median (range), years	2.7 (0–11.1)	0.0 (0–3.0)	2.7 (0.5–11.1)	3.0 (1.2–5.0)
SHAQ, median (range)	0.88 (0–2.63)	1.25 (0.63–2.63)	0.69 (0–1.71)	0.76 (0–1.50)
VAS Raynaud, median (range)	1.2 (0–2.95)	1.70 (0.30–2.70)	0.70 (0–2.95)	1.10 (0–2.40)
VAS digital ulcers, median (range)	0.54 (0–2.80)	0.00 (0–2.70)	0.20 (0–2.80)	0.00 (0–1.60)
VAS intestinal disease, median (range)	0.89 (0–2.80)	1.20 (0–2.70)	0.40 (0–2.80)	0.10 (0–1.70)
VAS breathing problems, median (range)	0.93 (0–2.90)	1.60 (0.70–2.90)	0.20 (0–2.80)	0.60 (0–1.20)
VAS general, median (range)	1.49 (0–2.90)	2.00 (0.60–2.90)	0.80 (0–2.80)	1.00 (0.50–1.20)
VAS pain, median (range)	0.98 (0–2.90)	1.30 (0.20–2.60)	0.20 (0–2.90)	0.75 (0–1.20)
EQ5D-5L index, median (range)	0.75 (0.04–0.96)	0.33 (0.04–0.73)	0.81 (0.40–1.00)	0.87 (0.71–0.92)

VAS scales ranges from 0 (no complaints) to 3 (severe complaints). EQ-5D-5L: EuroQoL 5 Dimensions 5 Levels; HSCT: haematopoietic stem cell transplantation; SHAQ: Scleroderma Health Assessment Questionnaire (range 0–3); VAS: visual analogue scale.

it with work, and therefore preferred an alternative therapy. Two other patients explained that they were disappointed after being informed about the expected outcomes. One of them thought that it was only worth the risks if HSCT could cure the condition. The other already had experienced improvement after initiating an alternative treatment and thought it was best to keep HSCT as a back-up plan.

Developing a frame of reference that facilitates decision-making: a difficult quest for knowledge

All patients received information from their rheumatologist and in most cases also from a haematologist and stem cell transplantation nurse. Most information was provided verbally. Patients were informed about the procedures, the expected outcomes, and potential adverse events and risks related to treatment in autologous HSCT in general.

Patients were in need of disease-specific information and personalized treatment risks and outcomes to make a decision. They appreciated that the information they received about the prognosis and risks of HSCT was clear and honest. We observed differences in attitudes towards treatment risks. Half of the patients who opted for HSCT thought that 10% treatment-related mortality was high, but still acceptable. The other half thought the risk was not high or did not know how to relate the risks to their own situation. Furthermore, many patients had positive expectations towards potential risks. Twelve patients, all males, believed they had lower treatment risks compared with other patients. Furthermore, two patients recounted that they had been told that HSCT is always successful, and without risks or complications. One patient could not recall being informed about treatment risks at all.

Reliable and SSc-specific patient information about treatment options was not available according to most patients. Two patients reported that they were sufficiently informed about the treatment options, and these were also patients who had participated in a clinical trial. There was no apparent relation between the rating of information provision and time since HSCT. Patients were discouraged by their rheumatologist to use the internet as an information source, because the reliability of the information could not be guaranteed. Some patients did search for additional information on the internet,

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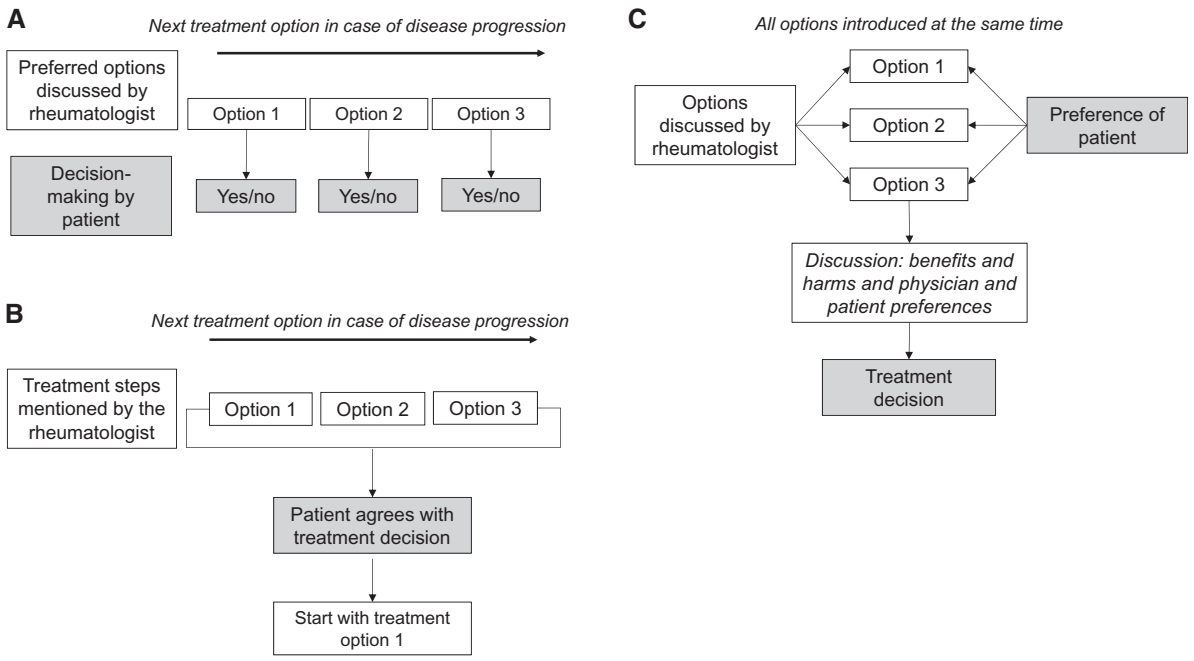
Fig. 1 Three approaches to discuss treatment options and making decisions



Three approaches were identified in discussion of treatment options with patients: (**A**) rheumatologist presents options sequentially; (**B**) a multi-step treatment plan was proposed; and (**C**) rheumatologist presents options concurrently.

although they had a difficult time finding reliable and understandable information.

More than half of the patients reported they could not oversee and balance all options, and that they lacked the information to be fully involved in the treatment decision. These patients preferred to be guided by their rheumatologist.

Patients who stated that they had made the decision themselves could better express their own decision-related health values in the interviews. Moreover, these patients felt sufficiently informed about the pros and cons to make this decision. Nine patients had had contact with a peer who shared their personal experiences about the treatment and decision-making process. This was highly valued as this contact provided more practical information and support than the patients had received from healthcare professionals.

Consultations around family planning and fertility preservation prior to therapy, in collaboration with the gynaecologist, were experienced as very distressing and difficult. Patients felt confronted with a sensitive aspect at a time at which they already had to make many tough decisions. They felt they did not have enough time to think about it, which rushed the decision with regard to HSCT and fertility preservation.

Physician-guided and entrusted decision-making

All patients thought the quality of the relationship and interaction with their rheumatologist was very important. Almost all patients wanted to know the treatment preference of their rheumatologist, and only two patients did not know what treatment their practitioner recommended. Ten patients felt that their rheumatologist made the ultimate decision, with no involvement of them.

The majority of patients described a paternalistic style of decision-making in which patients were strongly guided by their rheumatologist. One patient preferred to receive HSCT, but was directed towards another treatment by his rheumatologist. This led to feelings of rejection and fear, because he felt he did not have the chance to receive the optimal treatment.

Patients mentioned that trust was an important factor in accepting guidance from their rheumatologist. This trust was based on a longstanding relationship, the quality of the interaction or the good reputation of the rheumatologist. Having a good reputation was ascribed to having extensive experience or high scientific output. Some patients recounted that the recommended therapeutic plan was first discussed in a (multidisciplinary) team before it was introduced to them. These patients experienced this team effort as even more convincing than the individual preference of their rheumatologist. Interdisciplinary collaboration and alignment were valued highly.

Social interactions: protecting loved ones and feeling lonely

Social support during the decision-making process was very important to patients, although at the same time patients thought the interaction with their loved ones was complicated. Patients did not want to bother their

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Table 2 Themes, subthemes and issues important in the decision-making process, derived from the interviews

Themes	Subthemes	Issues
Poor prospects and low quality of life	Impact on daily life	Limitations in daily functioning
	Deterioration in health status	Failure of medication
		Fear of dying
	Shock about prognosis	Most invasive treatment seems the 'best option'
Expectations	Evaluation of different options	Feeling they could not see all options in perspective beforehand
		No other option provided by physician/team, different therapies offered one by one.
	Expectations of treatment outcome	Expectation that HSCT will stop the disease process
		Expectation that HSCT will cure the disease
		Being able to get back to work after HSCT
		Disappointment: HSCT cannot cure SSc
		No memory of expectations before treatment
	Expectations of side effects	Acceptable side effects given expected effects
		No expectations about side effects
		Lower risk of side effects due to good condition or young age
	Expectations of complications	More chance to have favourable outcome than other patients
		Ignore risks
		Symptoms are unbearable or quality of life is very low, so risks are acceptable
		Fear of dying, so risks are acceptable
	Employment	Loss of income, expectations that HSCT provides best chance to return to work
		Work defines identity, loss of work means loss of identity
		Work is a distraction from being ill
Knowledge	Information source	Trustworthiness of information on the internet
		Stories from peers: useful or not applicable to own situation
		Desire to read and understand scientific literature about HSCT and therapeutic options
Interaction with physician	Preference physician	Physician made treatment decision
		Physician tried to leave decision with patient
	Trust	Good interaction with physician and team
		Good reputation of physician (much experience/scientific output)
	Rejection	Exclusion for HSCT feels like rejection
Social interaction	Support from partner	Partner plays important role in decision-making and coping with emotions.
	Parents/family	Balancing sharing information, discussing risks
	Children	Children gave purpose during decision-making and treatment
		Difficulties telling children about condition and treatments
	Loneliness	Feeling misunderstood
		Struggling to cope with condition
		Feeling isolated
		Loss of friends

HSCT: haematopoietic stem cell transplantation.

family with concerns and believed that they had to make the decision and cope with the disease alone. Many patients reported feelings of loneliness at the very same time, also due to a dwindling social life and because they felt misunderstood by their family and friends. Some patients noticed that the illness put a strain on friendships and their relationship with their partner.

Patients also struggled with sharing information about the treatment and poor prognosis with their family and

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Table 3 Illustrative quotes from patients in the in-depth interviews

Decision-making approaches
P2: 'I was offered three treatment options: oral therapy (mycophenolate), monthly chemotherapy (cyclophosphamide) or autologous stem cell transplantation. I felt I had a choice.'
P4: 'They told me I could go for stem cell treatment and that I would die if I did not get this treatment; no alternatives were provided.'
P6: 'They mentioned that stem cell transplantation was an option, but I first had to try chemotherapy.'
Poor prospects and health status: leaving no other option
P6: 'I did not have a choice; my health was only getting worse. It was a sword of Damocles hanging above my head.'
P4: 'I was desperate, I was at war with myself.'
Expectations: maximizing chances for survival?
P3: 'I preferred to be treated with HSCT, because I read on the internet that this is the only treatment that could cure dcSSc.'
P11: 'I only would have taken the risk if HSCT could have cured the disease.'
P2: 'The most positive scenario would be stabilization of the disease.'
P4: 'The only thing I could think of was that I was going to die. If I did not choose HSCT, this was really going to happen.'
P3: 'I just want to get back to work, back to my normal life, get some distraction.'
Knowledge: the difficult quest for reliable and relevant information
P6: 'I was shocked to hear about chemotherapy; I thought this was only needed when you have cancer.'
P1: 'I was young and until recently in good health; I think I will have lower risks compared with older patients.'
P20: 'My rheumatologist told me not to Google, but of course I did Google. The information I found was confusing and did not make sense.'
P6: 'I found it very hard to apply the information to my own personal situation, which led to even more uncertainty and fear about what to expect.'
P19: 'Nobody really knows how it is and what to expect from stem cell transplantation; it really helped to talk to a peer.'
P16: 'It was hard to talk about fertility and family planning, about new life, while I was not even sure I would survive.'
Interaction with physician: guided decision-making
P1: 'My rheumatologist told me it was my decision and did not want to disclose his preferences. Yet, I preferred his opinion as an expert in this field.'
P3: 'My rheumatologist decided not to opt for HSCT. I was very upset about this. However, I believe it was the right decision eventually.'
P22: 'I fully relied on my doctors.'
P24: 'I always thought I was in good hands at this hospital. I experienced that a whole team of healthcare professionals was there to support me.'
Social interactions: involving loved ones and a tendency towards feeling lonely
P16: 'My family was very much involved; they supported me a lot.'
P2: 'I felt an obligation to my children to discuss the risks with them and to make the decision together.'
P8: 'I had to decide to continue with treatments because of my children. I could not leave them.'
P20: 'I thought it was more difficult to tell my children about the condition and prognosis than to hear and undergo the situation myself.'
P15: 'I wanted to protect my children, because there was so much uncertainty.'
P1: 'I used a booklet about stem cell transplantation in children to explain the procedure to my kids. They could read it themselves and look at the pictures, if they wanted more information.'
P2: 'Although I was surrounded by many people, I always felt alone.'

friends, especially with their children. Others consciously did not tell their children about the risks of treatments and treatment decision. Reasons not to inform children were that they did not want to worry them, or because they felt that it would negatively affect their role as a parent. Furthermore, when explaining the situation to their children, patients felt they really had to face the impact of dcSSc on their lives and the lives of their loved ones.

Some patients, however, involved their children in the decision-making process, and children were an important reason to opt for a treatment that carried both higher chances of benefit and more risk. For other patients, the high risk of HSCT was a reason not to go for this treatment. During the decision-making process, social contacts, especially partners, provided support and acted as a sounding board in so as far as they had been informed. Some patients shared their considerations and thoughts with their family, but made the decision themselves, while others weighed the opinion of others in their final decision.

Shared decision-making

Patients rated the decision-making process with a high score (80.64 out of 100, S.D. 15.6). There were no differences in rating between non-HSCT patients, HSCT patients and those scheduled for HSCT; or between age groups [$<$50 years: 80.9 (range 48.8–100.0) *vs* $\geq$50 years: 83.0 (range 62.2–100.0)]; males or females (mean 78.1 range 86.6–97.8 *vs* 86.7 range 59.9–100.0);

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or hospitals [University Medical Centre Utrecht: 89.9 (range 79.9–100.0), Radboud University Medical Centre 75.7 (range 48.8–100.0), Amsterdam Rheumatology & Immunology Centre, VU Medical Centre 82.1 (range 77.7–86.6), Leiden University Medical Centre 84.6 (range 62.6–97.8)]. We did not observe any trend between the approach of the rheumatologist in making the decision and the SDM rating. Three out of four patients that low-rated the decision-making process (SDQ-9 < 60), reported that they wished that HSCT had been performed earlier than it had been. All patients that were interviewed after HSCT or an alternative treatment, except for one, thought their decision was the right choice. This one person felt his QoL remained low, because his daily activities were very limited. The two patients that experienced a relapse post-HSCT did not regret their choice for HSCT.

Discussion

This study provides important insights into the treatment decision-making process in dcSSc from a patient's point of view. We identified several aspects that play an important role in SDM, including health status and prospects, expectations, knowledge, patient–physician interaction and social interaction. The patients in our study were generally satisfied with the process, which was mainly based on confidence in their rheumatologist. We found that patients perceived the decision-making as 'shared' overall, even though, for example, the rheumatologist steered them towards one or another option. Other studies made similar observations; patients defined the process as having been shared when they were satisfied with the interaction with their physician, notwithstanding that the decision had not been made conjointly [28]. Aspects like trust and feeling 'heard', might thus be more important for patients in appraising their involvement in decision-making than making the actual decision themselves or together with their rheumatologist. Nevertheless, patients in our study stressed essential SDM features, such as desiring more information, i.e. written information about alternative therapies, clear overview of pros and cons of therapies, what to expected after HSCT. They mentioned, additionally, that being made a partner in the decision contributed to trusting their rheumatologist. It was noteworthy that patients often did not mind being 'steered'. Implicit persuasion was previously shown to commonly occur in decision-making when more than one treatment option is available [29, 30]. Yet, the way in which patients are able to participate in decision-making differed per individual. The approach in which the patients are involved in the decision-making process should therefore be carefully tailored to the individual. Also, encouraging patients who were not forthcoming in becoming involved in decision-making had a positive effect on their satisfaction with the process [31]. Importantly, patients should not be forced to make the decision; ultimately it is for their rheumatologist to elicit enough information from patients to take into account the patients' values and preferences when balancing the pros and cons of treatment options.

In our study, patients mentioned a lack of SSc-specific information about treatment options as an impairment for SDM. Indeed, general information about HSCT in autoimmune diseases was published only recently [32], but is not fully applicable to dcSSc, as treatment risks are much higher. The health status at the time of decision-making had a major impact on how patients viewed information about HSCT. As patients further deteriorated, higher treatment risks were deemed more acceptable, which is in line with patients' evaluations in other conditions with a similar impact on QoL, such as stroke or [33] rheumatoid arthritis [34]. Also, the health status probably influenced the way rheumatologist explained the treatment options.

Secondly, fertility preservation had a huge emotional impact. In oncology, where similar experiences have been reported [35], guidelines have been developed in order to optimize timing of fertility counselling, and in this way optimizing treatment decision-making about oncological therapy [36]. Thirdly, patients' expectations about risks differed. Remarkably, male patients in our study believed they had lower risks of complications compared with other patients with dcSSc. This optimism bias might be a coping strategy, and is also described in other conditions, albeit not specifically in males [37]. To limit the extent of optimism bias, tailored information materials about risks of treatment-related complications could aid patients in assessing their personal risk. Currently, there are no such risk prediction scores for HSCT in dcSSc. Taken together, these observations

Table 4 Recommendations to improve the treatment decision-making process in dcSSc

Recommendations
A deliberate choice regarding the approach used to facilitate decision-making should be made
Clear, individualized information about the risks and benefits of all treatment options have to be provided at an early stage
The patient should be encouraged to participate in the decision-making process
The complex system of the patient (family, work, social life) and possible unmet needs in this system can be relevant to making the decision and should therefore be taken into account
Fertility and family planning needs to be adressed in advance and during follow-up
Contact with peers should be offered
Clear, SSc-specific information about HSCT and alternative treatments needs to be developed and provided
Research agenda
More insight in the perceptions of rheumatologists towards shared decision-making in dcSSc should be gained
Risk stratification models to provide patients with individualized information have to be developed

HSCT: haematopoietic stem cell transplantation.

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highlight the importance of SSc-specific and personalized patient education about HSCT, if possible at a time when they do not yet experience 'urgency' (advanced care planning) [38]. Based on our results, we formulated a number of recommendations for clinicians (see Table 4).

Our study has some limitations. For some patients, the decision-making process happened several years ago, which could lead to recall bias. Also, health status can influence the ability to recall the process correctly. Furthermore, the perception of the process could be influenced by their current condition. The small number of patients who are being offered HSCT in the Netherlands, however, strongly limited the possibility of including larger numbers of recently diagnosed patients. A strength of this study is that we included a relatively large and diverse group of patients.

In conclusion, our study shows that, in general, therapeutic decision-making in dcSSc where HSCT is considered is judged as shared by patients. Still this study also emphasizes that making therapeutic choices remains a challenge in dcSSc. We call for further research on how the attitude of rheumatologists governs the process of involving patients in decision-making in this population and how this might influence the patient. Secondly, studies should focus on the development of risk stratification models to provide patients with individualized information, and for SSc-specific information about HSCT, in order to further improve care.

Acknowledgements

We thank the patients for their time and valuable contributions to this research project. The preliminary results of our work were presented at the BSR Annual Conference 2019, abstract e070.

Funding: No specific funding was received from any funding bodies in the public, commercial or not-for-profit sectors to carry out the work described in this manuscript.

Disclosure statement: The authors have declared no conflicts of interest.

Supplementary data

Supplementary data are available at *Rheumatology* online.

References

1 Elhai M, Meune C, Boubaya M. Mapping and predicting mortality from systemic sclerosis. Ann Rheum Dis 2017;76:1897–905.

2 Ioannidis JP, Vlachoyiannopoulos PG, Haidich AB *et al*. Mortality in systemic sclerosis: an international meta-analysis of individual patient data. Am J Med 2005;118:2–10.

3 Fernandez-Codina A, Walker KM, Pope JE. Treatment algorithms for systemic sclerosis according to experts. Arthritis Rheumatol 2018;70:1820–8.

4 Burt RK, Shah SJ, Dill K *et al*. Autologous non-myeloablative haemopoietic stem-cell transplantation compared with pulse cyclophosphamide once per month for systemic sclerosis (ASSIST): an open-label, randomised phase 2 trial. Lancet 2011;378:498–506.

5 van Laar JM, Farge D, Sont JK *et al*. Autologous hematopoietic stem cell transplantation vs intravenous pulse cyclophosphamide in diffuse cutaneous systemic sclerosis: a randomized clinical trial. JAMA 2014;311:2490–8.

6 Sullivan KM, Goldmuntz EA, Keyes-Elstein L *et al*. Myeloablative autologous stem-cell transplantation for severe scleroderma. N Engl J Med 2018;378:35–47.

7 Kowal-Bielecka O, Fransen J, Avouac J *et al*. Update of EULAR recommendations for the treatment of systemic sclerosis. Ann Rheum Dis 2017;76:1327–39.

8 Denton CP, Hughes M, Gak N *et al*. BSR and BHPR guideline for the treatment of systemic sclerosis. Rheumatology 2016;55:1906–10.

9 Spierings J, van Rhijn-Brouwer FCC, van Laar JM. Hematopoietic stem-cell transplantation in systemic sclerosis: an update. Curr Opin Rheumatol 2018;30:541–7.

10 Sandman L, Munthe C. Shared decision-making and patient autonomy. Theor Med Bioeth 2009;30:289–310.

11 Joosten EA, DeFuentes-Merillas L, de Weert GH *et al*. Systematic review of the effects of shared decision-making on patient satisfaction, treatment adherence and health status. Psychother Psychosom 2008;77:219–26.

12 Harter M, Moumjid N, Cornuz J, Elwyn G, van der Weijden T. Shared decision making in 2017: international accomplishments in policy, research and implementation. Z Evid Fortbild Qual Gesundhwes 2017;123–124:1–5.

13 Marshall MN. Sampling for qualitative research. Fam Pract 1996;13:522–5.

14 Corbin JS. Basics of qualitative research: techniques and procedures for developing grounded theory, 3rd edn. Thousand Oaks: Sage, 2008.

15 Guest G, Bunce A, Johnson L. How many interviews are enough? An experiment with data saturation and variability. Field Methods 2006;18:59–82.

16 Charmaz K. Constructing grounded theory. A practical guide through qualitative analysis. London: Sage, 2006.

17 Rodenburg-Vandenbussche S, Pieterse AH, Kroonenberg PM *et al*. Dutch translation and psychometric testing of the 9-Item Shared Decision Making Questionnaire (SDM-Q-9) and Shared Decision Making Questionnaire-Physician Version (SDM-Q-Doc) in primary and secondary care. PLoS One 2015;10:e0132158.

18 Poole JL, Steen VD. The use of the Health Assessment Questionnaire (HAQ) to determine physical disability in systemic sclerosis. Arthritis Care Res 1991;4:27–31.

19 van Reenen MJ. EQ-5D-5L user guide. Rotterdam: EuroQoL Research Foundation, 2015.

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20 de Boer ME, Depla M, Wojtkowiak J *et al*. Life-and-death decision-making in the acute phase after a severe stroke: interviews with relatives. Pall Med 2015;29:451–7.

21 Bailey J. First steps in qualitative data analysis: transcribing. Fam Pract 2008;25:127–31.

22 Morgan DL. Practical strategies for combining qualitative and quantitative methods: applications to health research. Qual Health Res 1998;8:362–76.

23 Johnson SR, O'Brien KK. Qualitative methods in systemic sclerosis research. J Rheumatol 2016;43:1265–7.

24 Salt E, Peden A. The complexity of the treatment: the decision-making process among women with rheumatoid arthritis. Qual Health Res 2011;21:214–22.

25 Dierckx de Casterlé B, Gastmans C, Bryon E, Denier Y. QUAGOL: a guide for qualitative data analysis. Int J Nurs Stud 2012;49:360–71.

26 QRS International. NVivo qualitative data analysis software (computer program). v10, 2012. QRS International, Oss, the Netherlands. www.qsrinternational.com/nvivo/home (11 July 2019, date last accessed).

27 Tong A, Sainsbury P, Craig J. Consolidated criteria for reporting qualitative research (COREQ): a 32-item checklist for interviews and focus groups. Int J Qual Health Care 2007;19:349–57.

28 Kasper J, Heesen C, Kopke S, Fulcher G, Geiger F. Patients' and observers' perceptions of involvement differ. Validation study on inter-relating measures for shared decision making. PLoS One 2011;6:e26255.

29 Engelhardt EG, Pieterse AH, van der Hout A *et al*. Use of implicit persuasion in decision making about adjuvant cancer treatment: a potential barrier to shared decision making. Eur J Cancer 2016;66:55–66.

30 Karnieli-Miller O, Eisikovits Z. Physician as partner or salesman? Shared decision-making in real-time encounters. Soc Sci Med 2009;69:1–8.

31 van Stam MA, Pieterse AH, van der Poel HG *et al*. Shared decision making in prostate cancer care—encouraging every patient to be actively involved in decision making or ensuring the patient preferred level of involvement? J Urol 2018;200:582–9.

32 Jessop H, Farge D, Saccardi R *et al*. General information for patients and carers considering haematopoietic stem cell transplantation (HSCT) for severe autoimmune diseases (ADs): a position statement from the EBMT Autoimmune Diseases Working Party (ADWP), the EBMT Nurses Group, the EBMT Patient, Family and Donor Committee and the Joint Accreditation Committee of ISCT and EBMT (JACIE). Bone Marrow Transplant 2019;54:933–942.

33 Murtagh MJ, Burges Watson DL, Jenkings KN *et al*. Situationally-sensitive knowledge translation and relational decision making in hyperacute stroke: a qualitative study. PLoS One 2012;7:e37066.

34 Verburg RJ, Mahabali SD, Stiggelbout AM, Sont JK, van Laar JM. High dose chemotherapy and hematopoietic stem cell transplantation: a study of treatment preference in patients with rheumatoid arthritis and rheumatologists. J Rheumatol 2002;29:1653–8.

35 Jones G, Hughes J, Mahmoodi N *et al*. What factors hinder the decision-making process for women with cancer and contemplating fertility preservation treatment? Hum Reprod Update 2017;23:433–57.

36 Lee SJ, Schover LR, Partridge AH *et al*. American Society of Clinical Oncology recommendations on fertility preservation in cancer patients. J Clin Oncol 2006;24:2917–31.

37 Stoff BK, Swerlick RA. Reframing risk part II: methods for improving medical risk communication. J Am Acad Dermatol 2013;69:637–9.

38 Fahner JC, Beunders AJM, van der Heide A *et al*. Interventions guiding advance care planning conversations: a systematic review. J Am Med Dir Ass 2019;20:227–48.

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