Decision making for hematopoietic stem cell transplantation in pediatric, adolescent, and young adult patients with a hemoglobinopathy: shared or not?

Mekelenkamp, H.; Smiers, F.; Camp, N.; Stubenrouch, F.; Lankester, A.; Vries, M. de

Received: 4 February 2021 | Revised: 16 April 2021 | Accepted: 19 April 2021

DOI: 10.1002/pbc.29099

Pediatric Blood & Cancer

TRANSPLANTATION: RESEARCH ARTICLE

Decision making for hematopoietic stem cell transplantation in pediatric, adolescent, and young adult patients with a hemoglobinopathy—Shared or not?

Hilda Mekelenkamp[1,2] | Frans Smiers[1] | Nomie Camp[1] | Fabienne Stubenrouch[3] | Arjan Lankester[1] | Martine de Vries[1,2]

[1] Willem-Alexander Children's Hospital, Department of Pediatrics, Leiden University Medical Centre, Leiden, The Netherlands

[2] Department of Medical Ethics and Health Law, Leiden University Medical Centre, Leiden, The Netherlands

[3] Department of Surgery, Amsterdam UMC, location AMC, Amsterdam, The Netherlands

Correspondence
Hilda Mekelenkamp, Willem-Alexander Children's Hospital, Department of Pediatrics, Leiden University Medical Centre, Leiden, The Netherlands.
Email: H.Mekelenkamp@lumc.nl

On behalf of the SCORE consortium.

Abstract

Background: Hematopoietic stem cell transplantation (HSCT) offers an established curative option for sickle cell disease (SCD) and thalassemia patients but is associated with significant risks. Decision making is a complex process and shared decision making (SDM) could be a fitting approach in case of such preference-sensitive decisions. This study investigated what level of SDM is used in conversations with hemoglobinopathy patients and/or their caregivers considering HSCT as a curative treatment option.

Methods: Longitudinal, descriptive study using the Observing-Patient-Involvement-in-Decision-Making scale (OPTION^5) scale to determine the level of SDM in conversations with 26 hemoglobinopathy patients and/or their caregivers.

Results: The total mean OPTION^5 score was 43%, which is a moderate SDM approach. There was no difference between conversations with thalassemia patients and SCD patients. Conversations needing an interpreter scored worse than nontranslated conversations. The best scoring OPTION^5 item was item 3: "informing about the various treatment options" (mean score 2.3 on scale 0–4). For OPTION^5 item 4: "eliciting patients' preferences" a more skilled effort was measured for SCD patients compared to thalassemia patients.

Conclusions: The mean OPTION^5 score of "moderate" was achieved mainly by giving information on available options, which is primarily a one-way communication. The SDM process can be improved by actively inviting patients to deliberate about options and including their elicited preferences in decision making.

KEYWORDS
hematopoietic stem cell transplantation, hemoglobinopathy, shared decision making, sickle cell disease, thalassemia

1 | INTRODUCTION

Hemoglobinopathies are one of the most common genetic diseases that affect humans worldwide. For the scope of this study

Abbreviations: HCP, health care professionals; HSCT, hematopoietic stem cell transplantation; OPTION, Observing-Patient-Involvement-in-Decision-Making scale; SCD, sickle cell disease; SDM, shared decision making.

hemoglobinopathy will be defined as the two most prevalent forms, sickle cell disease (SCD) and beta-thalassemia. Both, SCD and transfusion-dependent beta-thalassemia are chronic invalidating diseases with a large impact on quality of life, life expectancy, and overall health. Despite advanced supportive treatment, including hydroxyurea, SCD patients suffer from painful vaso-occlusive crises, cumulative organ damage, and anemia. These complications vary per patient but can lead to chronic blood or exchange transfusion. The supportive treatment demands adherence to a strict regime.[1,2] For patients with beta-thalassemia, the main supportive treatment includes frequent blood transfusions with risks of allo-immunization and iron overload necessitating chelation therapy.[3,4] Hematopoietic stem cell transplantation (HSCT) offers an established curative option for both these groups of hemoglobinopathy patients. However, HSCT carries the risk of graft-versus-host disease, graft failure, and organ damage. In some patients these complications can be lethal. Somewhere in their treatment trajectory, patients may be offered the curative option of HSCT.[5–7] The timing of coming in contact with the possibility of HSCT varies from early after diagnosis in infancy to somewhere during adulthood. Once familiar with the option, patients and/or their caregivers have a choice: either to choose for HSCT or to continue supportive, noncurative treatment. The recent availability of gene therapy for hemoglobinopathies is emerging as another curative option,[8] rendering decision making even more complex.

Within medical decision making, two types of decisions have been described.[9] First, an "effective" decision in which a scientific certainty exists, and clearly more pros than cons are known. Second, a "preference-sensitive" decision, wherein no clear-cut answers are available and the pros and cons are dependent on individual values.[9] Shared decision making (SDM) is the preferred model to involve patients in thinking along with the health care professionals (HCP) about preference-sensitive decisions.[10,11] The SDM process initially was described as a three-step model[10] and is further developed as a four-step model.[11] First, the HCP makes clear that a decision has to be made; second, information is provided about the available options with their advantages and disadvantages. Third, the preferences of the patient are explored and finally an appointment is made when and how the decision will be taken. A meta-analysis showed that SDM in pediatrics improved knowledge, reduced decisional conflict, and could lead to increased satisfaction.[12] The question is whether and to what extent an SDM approach is used in current practice when considering an HSCT in circumstances in which there is no clear-cut answer as to what the best treatment is, such as for hemoglobinopathy patients. This study investigates the level of SDM used in conversations with hemoglobinopathy patients and/or their caregivers considering HSCT as a curative treatment option. This insight can support the use of SDM principles in clinical practice.

2 | METHODS

2.1 | Design

This longitudinal descriptive study is part of a larger prospective, qualitative study with 27 families, focusing on the HSCT decision-making process for hemoglobinopathy patients. The research ethics committee of the Leiden University Medical Centre approved the study protocol (P17084).

2.2 | Sample

Patients with SCD or transfusion-dependent thalassemia and/or their caregivers who had a conversation with their hematologist and/or HSCT specialist about a possible HSCT were selected. Patients between 0 and 35 years and/or their caregivers were eligible for inclusion. Patients or parents who did not consent were excluded. Patients were recruited from Dutch specialized medical centers for hemoglobinopathies. The local staff identified eligible patients and obtained written informed consent from the patients and/or their legal representatives.

2.3 | Data collection

Data collection consisted per patient of one or two audio-recorded conversations with their referring physician and/or with the HSCT specialist combined with sociodemographic characteristics. Conversations took place on the initiative of a physician or the patient/family to discuss the option of HSCT. Caregivers were always involved in situations with patients below 16 years old. For patients above the age of 16 years, parent's involvement depended on the patients' preferences. In the event of a language barrier, the conversation was, live or by phone, translated by a professional interpreter.

2.4 | Level of SDM

The level of SDM in conversations was measured by using the Observing-Patient-Involvement-in-Decision-Making scale (OPTION^5 scale).[13,14] This well-established and reliable instrument assesses mainly the clinicians' behavior in clinical conversations from an observer perspective. The OPTION^5 was chosen because it differentiates better between various levels of patient involvement compared to the more extensive 12-item OPTION scale.[15] Each recorded conversation was graded on the following five aspects: addressing the existence of alternate treatment options (item 1), supporting the patients in deliberation (item 2), informing about the various treatment options (item 3), eliciting the patient's preferences (item 4), and integrating the patient's preferences as decisions are made (item 5). Every item was scored on a 5-point Likert scale from 0 to 4 in which 0 indicates no effort was made whereas 4 indicates exemplary effort (Table 1).[16]

2.5 | Data analysis

All conversations were transcribed verbatim and scored according to the OPTION^5 manual[16] independently by two study team members (Hilda Mekelenkamp, Nomie Camp) after following an online

TABLE 1 OPTION[5] items

OPTION[5] items
Item 1
For the health issue being discussed, the clinician draws attention to or confirms that alternate treatment or management options exist or that the need for a decision exists. If the patient rather than the clinician draws attention to the availability of options, the clinician responds by agreeing that the options need deliberation. *0 = No effort, 1 = Minimal effort, 2 = Moderate effort, 3 = Skilled effort, 4 = Exemplary effort*
Example:"I will give you an explanation as to what HSCT entails and which options are available for you. Afterwards, we have to come to a decision together." (Case 8)
Item 2
The clinician reassures the patient or reaffirms that the clinician will support the patient to become informed or deliberate about the options. If the patient states that they have sought or obtained information prior to the encounter, the clinician supports such a deliberation process. *0 = No effort, 1 = Minimal effort, 2 = Moderate effort, 3 = Skilled effort, 4 = Exemplary effort*
Example:"I would like to explain you what it entails to undergo a transplantation, how the treatment works, and what the associated risks are, so that we can then decide together whether we want to consider this as a possibility." (Case 20)
Item 3
The clinician gives information or checks understanding about the options that are considered reasonable (this can include taking no action), to support the patient in comparing alternatives. If the patient requests clarification, the clinician supports the process. *0 = No effort, 1 = Minimal effort, 2 = Moderate effort, 3 = Skilled effort, 4 = Exemplary effort*
Example:"I will explain what a transplantation is, what the possibilities are, what can or cannot be done, when you should or should not do it. All the pros and cons [*explaining supportive care, HSCT, gene therapy*]. But I do think it is fair to explain this last possibility is developing because it is an important decision that you have to make, whether or not to transplant. And then you must be well informed with all the information." (Case 1)
Item 4
The clinician makes an effort to elicit the patient's preferences in response to the options that have been described. If the patient declares their preference(s), the clinician is supportive. 0 = *No effort, 1 = Minimal effort, 2 = Moderate effort, 3 = Skilled effort, 4 = Exemplary effort*
Example:"Now that I have explained everything, what are your thoughts when you compare everything and what you have read? What is your impression so far?" (Case 15)
Item 5
The clinician makes an effort to integrate the patient's elicited preferences as decisions are made. If the patient indicates how best to integrate their preferences as decisions are made, the clinician makes an effort to do so. *0 = No effort, 1 = Minimal effort, 2 = Moderate effort, 3 = Skilled effort, 4 = Exemplary effort*
Example:"Well, we certainly do not have to decide right now. We can decide to discuss this further together in a few weeks, then you have had the time to think about it again, to talk about it at home. Perhaps some new questions will have come up, that is possible and then you can write them down." (Case 8)

course developed by the authors of the OPTION[5] scale.[17] Furthermore, three conversations were scored together with an external team, experienced in using the OPTION[5] scale.[15,18] In order to support consistent scoring, the raters discussed on a regular basis how to apply the OPTION[5] scale for the conversations in this study, and an inter-rater reliability was calculated with a kappa value after sets of five to 10 conversations to guide this discussion. The raters discussed all outcomes and consensus was reached for every individual item and conversation. The OPTION[5] scores were added up to a total score between 0 and 20 for every individual conversation. In case two conversations were recorded for an individual patient, the decision process was scored on *patient-level* by determining the overall total score by the sum of the highest achieved items scores across both conversations. The total scores were rescaled to 100 and expressed as a percentage for a clear presentation and easier interpretation of the data. For patients with thalassemia, SCD, transplanted patients, and those on supportive care, the total mean OPTION score, SD, range, and median item scores were reported on *patient-level* ($N = 26$ patients). For translated, nontranslated conversations, conversations by a referring hematologist, and by an HSCT specialist, the total mean OPTION score, SD, range, and median item scores were reported on *conversation-level*. Differences between these subgroups were tested using the likelihood ratio test for ordinal regression.

3 | RESULTS

Twenty-six patients were included between June 2017 and June 2019. All patients had at least one conversation with an HSCT specialist; we missed one recording of a patient with the HSCT specialist. For 12 patients one conversation was recorded, for the remaining 14 patients two conversations were recorded, resulting in 40 conversations. The conversations were mostly ($n = 34/40$) performed by an HSCT specialist; for some patients ($n = 6/40$), a conversation with their referring hematologist was also recorded. For five patients, the HSCT specialist was also their hematologist. In 10/40 conversations,

TABLE 2 Characteristics

Characteristics	Total number of included patients (*N* = 26)	Number of sickle cell disease patients (*N* = 18)	Number of thalassemia patients (*N* = 8)
Patient's sex			
Female	16	12	4
Male	10	6	4
Patient's age (years)			
<12	14	8	6
12–16	6	5	1
>16	6	5	1
Transplanted			
Yes	20	13	7
No	6	5	1
Conversations (*N* = 40)			
With hematologist	6	6	0
With HSCT specialist	34	21	13
Including interpreter	10	2	8

a professional interpreter was present. The majority of the patients (18/26) had SCD and 14/26 patients were younger than 12 years old. All patients had a solid indication for HSCT. Twenty patients chose and underwent an HSCT. The remaining six patients refrained from the HSCT option; one due to personal circumstances not compatible with HSCT, one due to unavailability of a family-acceptable donor, and four because of a deliberate choice against HSCT (Table 2 and Figure 1). The patients were referred by nine Dutch hospitals to two national HSCT referral centers for adult and pediatric patients. Both HSCT centers are specialized in the care for hemoglobinopathy patients. Twelve specialists were involved, and the mean duration of conversations was 56 minutes (range 16–105). Nontranslated conversations had a mean duration of 50 minutes (range 16–77), whereas translated conversations had a mean duration of 74 minutes (range 41–105). During the 40 conversations, 21 patients were involved, 33 caregivers, five siblings (including three potential donors), and five other relatives.

3.1 | Level of SDM

The total mean score of the SDM process within one or two conversations in 26 patients was 43% (SD 11%, range 20–60), meaning a moderate effort.

The individual OPTION^5 items 1, 2, 4, and 5 scored on a scale from 0 to 4, a median score between 1 and 2, corresponding with minimal-moderate effort. The highest score of 2.35 was measured for OPTION^5 item 3, informing about the various treatment options, meaning a moderate-skilled effort. The distributions of the different OPTION^5 items are shown in Figure 2A. "Exemplary effort" was not measured in any OPTION^5 item, "no-effort" was measured once in OPTION^5 items 1 and 2, and four times in OPTION^5 item 4.

Translated conversations (*N* = 10) had a total mean SDM score of 36% (SD 11%; range 20–50) compared to 39% (SD 12%; range 10–60) for the nontranslated conversations (*N* = 30), measured *on conversation level*. When comparing individual OPTION^5 items in translated conversations with nontranslated conversations, no statistically significant differences were found. Comparable results were measured for item 1, addressing the existence of alternate treatment options, and item 5 integrating the patient's preferences (Figure 2B,C). OPTION^5 item 2, supporting the patients in deliberation, scored more often a moderate or skilled effort in translated conversations (median 2) compared to nontranslated conversations (median 1). OPTION^5 item 3, informing about the various treatment options, scored in translated conversations primarily 2, moderate effort (9/10), whereas in the nontranslated conversations this item scored mainly 2, moderate (14/30) and 3, skilled effort (11/30). In translated conversations compared to nontranslated conversations OPTION^5 item 4, eliciting the patient's preferences, scored both with a median of 1 (minimal effort) and in four of 10 in translated conversations and in seven of 30 of the nontranslated conversations no effort was measured.

Conversations with referring hematologists (*N* = 6, mean SDM 39%, SD 7) compared to conversations performed by HSCT specialists (*N* = 34, mean SDM 38%, SD 12) scored significant differences for OPTION^5 items 3 and 4. Item 3 scored higher with a median 2 in HSCT specialists compared to a median of 1.5 in referring hematologists (*p*-value .007). Item 4 scored the opposite, where referring hematologists scored higher with a median score of 2.5 compared to a median score of 1 for HSCT specialists (*p*-value .010). OPTION^5 items 1 and 2 scored comparably low and item 5 scored higher in referring hematologists, but not significantly different (Figure 2D,E).

Conversations with thalassemia patients (*N* = 8) and SCD patients (*N* = 18) had comparable mean SDM scores of 44% (SD11%; range 30–55) and 43% (SD11%; range 20–60), respectively, measured *on patient level*. Conversations with thalassemia patients and SCD patients

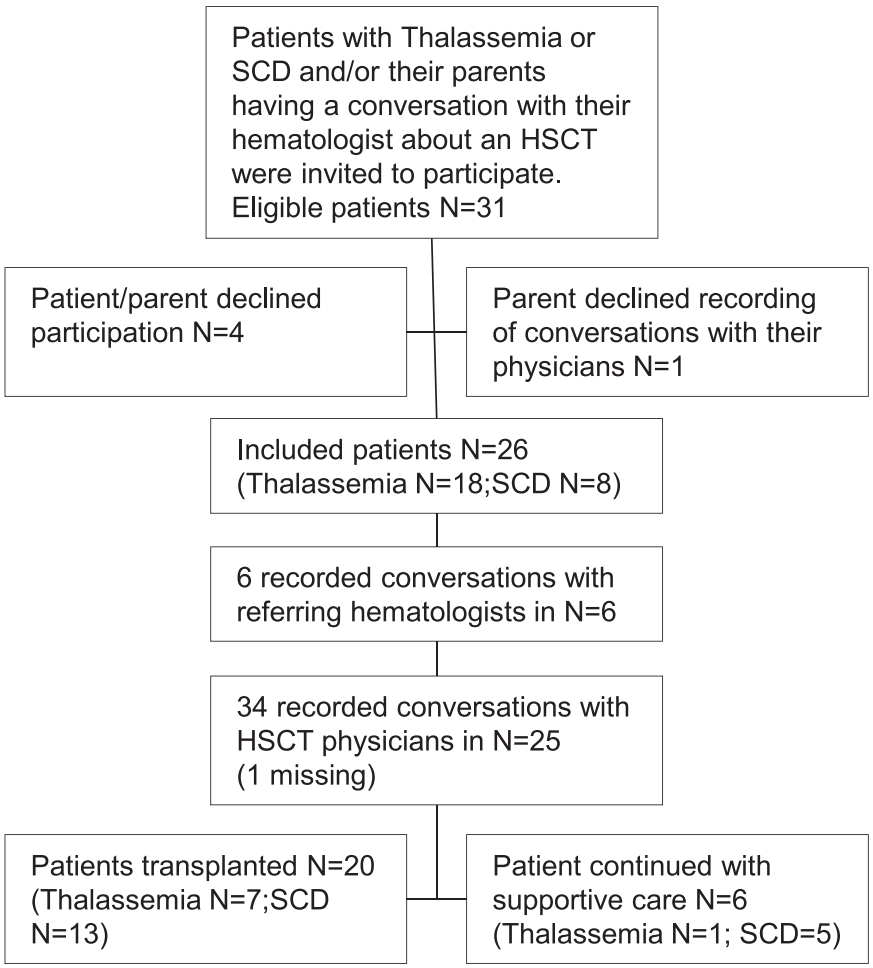

FIGURE 1 Inclusion procedure. HSCT, hematopoietic stem cell transplantation; SCD, sickle cell disease

scored comparably for OPTION^5 items 1 and 3 (Figure 2F,G). OPTION^5 items 4, eliciting the patient's preferences and item 5, integrating the patient's preferences, showed higher median scores in SCD patients of 2, compared to 1 in thalassemia patients. A significant difference between SCD patients and thalassemia patients was measured for OPTION^5 item 2, supporting the patients in deliberation, with a median score of 1 for SCD patients and 2 for thalassemia patients (p-value .011).

In conversations with patients who eventually were transplanted (N = 20), the total mean SDM score was 43% (SD 11) compared to 46% (SD 9) in patients remaining on supportive care (N = 6). Conversations with patients who remained on supportive care had higher median scores for the different OPTION^5 items (Figure 2H,I), but not significantly different.

4 | DISCUSSION

In this study, we show that our current SDM process in conversations with patients and families about HSCT as a curative option for hemoglobinopathies achieved a moderate score of 43%.

OPTION^5 item 3, informing about the various treatment options, was the best scoring part of SDM in the conversations. This item scored with a mean of 2.35 (moderate to skilled effort) and could not be scored with exemplary effort. Furthermore, we observed that detailed information was provided about the HSCT, but less on the option of continuing with supportive care, with its pros and cons. This relatively high score for OPTION^5 item 3 could be explained by the way the care path for hemoglobinopathy patients evolves. On the initiative of a physician or patient, a focused conversation about a possible HSCT takes place. SDM in the current care path of hemoglobinopathy patients seems to focus mainly on providing information on the possibilities of HSCT, whereas a more comprehensive SDM process can be achieved by including all treatment possibilities.

The other OPTION^5 items scored on average less than 2, corresponding to moderate effort or less. We observed that OPTION^5 item 1 (addressing the existence of alternate treatment options) was included at the beginning of the conversation in only a third of the conversations. The beginning of the conversation is the best moment to actively involve patients in the SDM process. OPTION^5 item 1 does not differentiate for this timing, suggesting that this item easily could be scored too high and in reality SDM on this aspect could be even lower. The

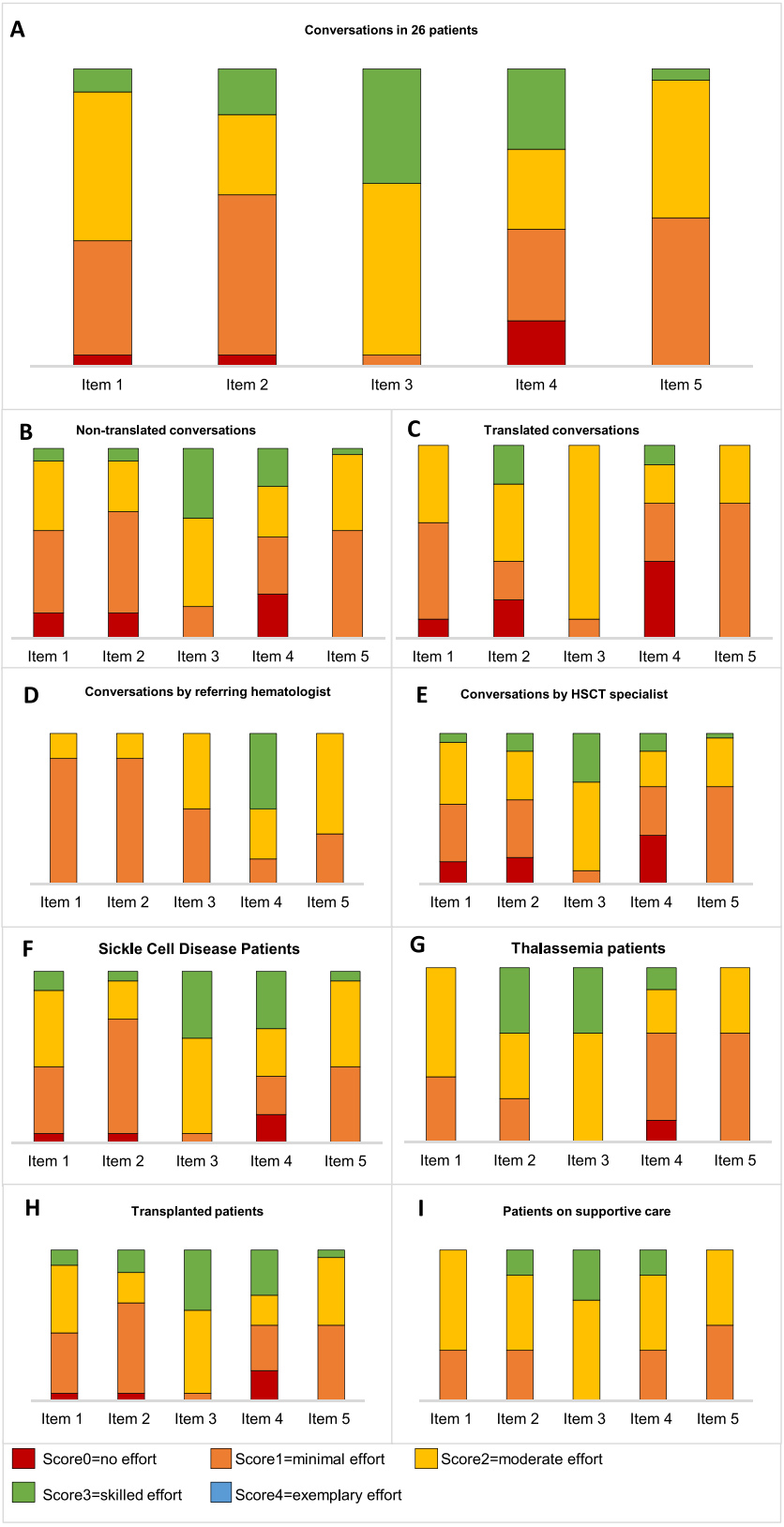

FIGURE 2 Distribution of shared decision making (OPTION[5]) items. (A) Distribution of the OPTION[5] items in conversations in 26 patients on patient level*. (B) Distribution of the OPTION[5] items in nontranslated conversations ($N = 30$) on conversation level. (C) Distribution of the OPTION[5] items in translated conversations ($N = 10$) on conversation level. (D) Distribution of the OPTION[5] items in conversations by a referring hematologist ($N = 6$) on conversation level. (E) Distribution of the OPTION[5] items in conversations with HSCT specialists ($N = 34$) on conversation level. (F) Distribution of the OPTION[5] items in conversations in sickle cell disease patients ($N = 18$) on patient level*. (G) Distribution of the OPTION[5] items in conversations in Thalassemia patients ($N = 8$) on patient level*. (H) Distribution of the OPTION[5] items in conversations in transplanted patients ($N = 20$) on patient level*. (I) Distribution of the OPTION[5] items in conversations in patients on supportive care ($N = 6$) on patient level*
*In case two conversations were recorded for an individual patient, the decision process was scored on patient level by determining the overall total score. Item 1 = addressing the existence of alternate treatment options; item 2 = supporting the patients in deliberation; item 3 = informing about the various treatment options; item 4 = eliciting the patient's preferences; item 5 = integrating the patient's preferences as decisions are made

relatively low scores for OPTION[5] items 1, 2, 4, and 5 could be explained by the ways physicians were used to give information on HSCT. Traditionally, HSCTs were reserved for patients in which alternative treatment options had less chance of cure and therefore HSCT complications were more accepted, as in oncology treatment. In that context, parents did not experience treatment decisions as a real choice, but rather as a protocolized step focusing on their child's cure.[19,20] It is imaginable that therefore HSCT decision-making conversations were not that much focused on SDM but rather on informing, as illustrated by existing tools.[21,22] In recent years however, the indications for HSCT have become broader and more patients with hemoglobinopathies are transplanted.[23] Decision making for hemoglobinopathy patients seems different and highly dependent on patients' own perspectives.[24–27]

Compared to the nontranslated conversations, the translated conversations had relatively lower scores for items 3 and 4 (Figure 2), which was most pronounced for item 3. The differences for a score 2 or 3 rely on the effort for checking the understanding. It could be hypothesized that translated conversations demand a different effort, which when absent may easily disturb the process of SDM.

In thalassemia patients we observed less attention to OPTION[5] items 4 and 5, eliciting and integrating patients' preferences. In patients with transfusion-dependent thalassemia, the option of HSCT is more established than in SCD patients. As a consequence, informing the family on the transplant option more readily takes the structure of an effective rather than a preference-sensitive decision.[4] Once gene therapy becomes feasible as a second curative treatment option, an SDM approach becomes even more important. This also applies to improved chelation therapy, which may diminish the negative side effect of chronic erythrocyte transfusions.[28] OPTION[5] item 2, supporting the patient in becoming informed, scored in two-thirds of the SCD patients no-minimal effort, compared to two-thirds of thalassemia patients with moderate-skilled effort. The opposite in these subgroups applied for items 4 and 5 with less skilled effort in thalassemia patients compared to SCD patients. This effect could be related; when less effort is put into eliciting preferences, more time and attention can be used to inform the patient.

Differences in conversations between referring hematologists and HSCT physicians were most obvious for OPTION[5] items 3 and 4. Item 3 scored significantly higher in conversations with HSCT specialists, pointing to the informative character of the conversations HSCT specialist have with the patients, mostly after referral by a hematologist. In contrast, conversations with the referring hematologists scored significantly higher for item 4. This could be explained by the often longer existing relationship in which it is the hematologist's responsibility to guide the patient through their illness process.

Compared to other SDM studies, the overall mean SDM score in our study of 43% compares favorably. While studies using OPTION[5] in unmanipulated routine clinical conversations scored the SDM process with a range between 11 and 82, most studies had a mean SDM score of below 40.[15,18,29–35] A review assessing SDM with the OPTION[12] found a mean score of 23, whereas only 38% of the studies reported scores above or equal to their used cut-off point of 25.[36] The best scoring OPTION[5] item was variable among these studies.[15,18,29–35]

The results of our study showed a moderate SDM approach, indicating that HSCT decision making for hemoglobinopathy patients is approached as a preference-sensitive decision.[9] However, the fact that this result was mainly influenced by the focus on informing (OPTION[5] item 3), and the wide range of SDM (20–60%) suggests that the HSCT decision making was approached ambiguously, as either a preference-sensitive or an effective decision. This phenomenon has previously been reported in a qualitative study in SCD patients, describing two different approaches of disease-modifying therapies, including HSCT.[37] Physicians used a more proponent approach in case of severe disease rather than a collaborative approach. Besides disease severity, intensity of treatment and urgency of treatment appeared to be influencing factors.

The results of the current study highlight the need to improve the SDM process about curative options for hemoglobinopathy patients. Based on our results, all OPTION[5] items deserve more attention in conversations, particularly by inviting patients in deliberating the options and in eliciting and integrating their preferences. To achieve this, first, the skill how to apply SDM in clinical conversations should be given with simulated patient encounters and by reflecting on personal skills.[11,38–40] Second, given our observation that conversations were mainly focused on HSCT, detailed information needs to be given about all possible treatment options, including HSCT, possible future gene therapy, and also continuation of supportive care. Third, using sufficient time is important and not only as resource. As stated by Pieterse et al.[41] "a minute spent in giving information may turn out to be less important than a minute waiting silently for questions….." Different ways of informing can be used; a substantial part of the technical HSCT information can be provided using other sources, like videos. The time during the consultations can be used to talk about the understanding and customization of this information. A significant part of SDM exists of eliciting the patient's preferences and values and incorporating these in the decision. This implicates that the conversations should continue after providing information focused on the patients' considerations. The question arises who should do this. Therefore, as a fourth step to improve SDM about curative options for hemoglobinopathy patients, it is recommended to make clear what parts of the treatment possibilities and which SDM steps will be discussed by which HCP: the hematologist, HSCT specialist, or another involved HCP, such as a nurse specialist.[42] This suggests that the focus of the different conversations may vary, and it demands a continuous interdisciplinary collaboration focused on the SDM process of the specific patient. Finally, patients can be supported with the use of decision aids with information,[22] assistance in forming their preferences, and with pre-scripted questions to ask during conversations.[11,36]

This descriptive analysis of the SDM process provides new insights in the current practice of hemoglobinopathy patients. Strength of our study is that we analyzed the decision-making process from an objective observer perspective within one or two conversations performed by different physicians, including translated conversations. However, it remains a subjective interpretation of a conversation. We maximized

objectivity by double, independent coding and involving an external team. We chose to differentiate in our analysis between SCD and thalassemia, as well as between translated and nontranslated conversations. We did not do this for cultural background. Our sample was too heterogenous to be able to do so, but most importantly we aimed for an objective evaluation of the SDM process. A variable like cultural background would give a subjective interpretation. Both groups of translated and nontranslated conversations consisted of families with different nationalities, as most patients with hemoglobinopathies are not of Dutch origin. Although not all families were Dutch native speakers, not all the conversations needed translation. The included conversations in our study can be biased due to its focus on HSCT, resulting in mainly conversations in an HSCT center. Therefore, we could have missed parts of the decision-making process, even more because talking about treatment can be a process of years in the studied population. We scored SDM in the conversations by reading transcripts, where most studies used audiotaped conversations. Minor details as intonations could easily be missed in transcripts, leading to different ratings. However, transcript makes it easier to re-read sentences and provide a more precise rating.

In conclusion, the mean OPTION^5 score of "moderate" was achieved mainly by giving information on available options, which is primarily a one-way communication. The SDM process can be improved by actively inviting patients to deliberate about the treatment options and including patients' preferences in final decision making. More insight into patients' considerations regarding the decision-making process can be supportive in order to actively include these perspectives in the conversations and to design decision aids for patients. A qualitative study is ongoing, aiming to provide insight into these considerations.

ACKNOWLEDGMENTS

We thank all the patients, their families, and the involved professionals for their contribution to this study.

CONFLICT OF INTEREST

The authors declare that there is no conflict of interest.

DATA AVAILABILITY STATEMENT

The data that support the findings of this study are available from the corresponding author upon reasonable request.

ORCID

Hilda Mekelenkamp https://orcid.org/0000-0002-4410-1845
Frans Smiers https://orcid.org/0000-0002-8131-3521
Arjan Lankester https://orcid.org/0000-0002-6703-2993
Martine de Vries https://orcid.org/0000-0002-3178-7470

REFERENCES

1. Meier ER, Rampersad A. Pediatric sickle cell disease: past successes and future challenges. *Pediatr Res*. 2017;81(1-2):249-258.
2. Hulbert ML, Shenoy S. Hematopoietic stem cell transplantation for sickle cell disease: progress and challenges. *Pediatr Blood Cancer*. 2018;65(9):e27263.
3. Origa R. β-Thalassemia. *Genet Med*. 2017;19(6):609-619.
4. Peters C. Allogeneic hematopoietic stem cell transplantation to cure transfusion-dependent thalassemia: timing matters! *Biol Blood Marrow Transplant*. 2018;24(6):1107-1108.
5. King A, Shenoy S. Evidence-based focused review of the status of hematopoietic stem cell transplantation as treatment of sickle cell disease and thalassemia. *Blood*. 2014;123(20):3089-3094. quiz 3210.
6. Khemani K, Katoch D, Krishnamurti L. Curative therapies for sickle cell disease. *Ochsner J*. 2019;19(2):131-137.
7. Angelucci E, Matthes-Martin S, Baronciani D, et al. Hematopoietic stem cell transplantation in thalassemia major and sickle cell disease: indications and management recommendations from an international expert panel. *Haematologica*. 2014;99(5):811-820.
8. Thompson AA, Walters MC, Kwiatkowski J, et al. Gene therapy in patients with transfusion-dependent β-thalassemia. *N Engl J Med*. 2018;378(16):1479-1493.
9. Wennberg JE. Dealing with medical practice variations: a proposal for action. *Health Aff (Millwood)*. 1984;3(2):6-32.
10. Elwyn G, Frosch D, Thomson R, et al. Shared decision making: a model for clinical practice. *J Gen Intern Med*. 2012;27(10):1361-1367.
11. Stiggelbout AM, Pieterse AH, De Haes JC. Shared decision making: concepts, evidence, and practice. *Patient Educ Couns*. 2015;98(10):1172-1179.
12. Wyatt KD, List B, Brinkman WB, et al. Shared decision making in pediatrics: a systematic review and meta-analysis. *Acad Pediatr*. 2015;15(6):573-583.
13. Elwyn G, Tsulukidze M, Edwards A, Legare F, Newcombe R. Using a 'talk' model of shared decision making to propose an observation-based measure: observer OPTION 5 Item. *Patient Educ Couns*. 2013;93(2):265-271.
14. Barr PJ, O'Malley AJ, Tsulukidze M, Gionfriddo MR, Montori V, Elwyn G. The psychometric properties of Observer OPTION(5), an observer measure of shared decision making. *Patient Educ Couns*. 2015;98(8):970-976.
15. Stubenrouch FE, Pieterse AH, Falkenberg R, et al. OPTION(5) versus OPTION(12) instruments to appreciate the extent to which healthcare providers involve patients in decision-making. *Patient Educ Couns*. 2016;99(6):1062-1068.
16. Elwyn G, Grande SW, Barr P. *Observer OPTION5 Manual 2018*. 2018.
17. Paradis Montibello R, Nye A, Elwyn G. *Online, Self-Paced Training for Observer OPTION 5*. 2017. http://www.glynelwyn.com/observer-option-5-2014.html. Accessed November 8, 2019.
18. Stubenrouch FE, Mus EMK, Lut JW, Hesselink EM, Ubbink DT. The current level of shared decision-making in anesthesiology: an exploratory study. *BMC Anesthesiol*. 2017;17(1):95.
19. Pentz RD, Pelletier W, Alderfer MA, Stegenga K, Fairclough DL, Hinds PS. Shared decision-making in pediatric allogeneic blood and marrow transplantation: what if there is no decision to make? *Oncologist*. 2012;17(6):881-885.
20. Mekelenkamp H, Lankester AC, Bierings MB, Smiers FJW, de Vries MC, Kars MC. Parental experiences in end-of-life decision-making in allogeneic pediatric stem cell transplantation: "Have I been a good parent?" *Pediatr Blood Cancer*. 2020;67(5):e28229.
21. Hankins J, Hinds P, Day S, et al. Therapy preference and decision-making among patients with severe sickle cell anemia and their families. *Pediatr Blood Cancer*. 2007;48(7):705-710.
22. Krishnamurti L, Ross D, Sinha C, et al. Comparative effectiveness of a web-based patient decision aid for therapeutic options for sickle cell disease: randomized controlled trial. *J Med Internet Res*. 2019;21(12):e14462.
23. Passweg JR, Baldomero H, Basak GW, et al. The EBMT activity survey report 2017: a focus on allogeneic HCT for nonmalignant indications and on the use of non-HCT cell therapies. *Bone Marrow Transplant*. 2019;54(10):1575-1585.

24. Nickel RS, Kamani NR. Ethical challenges in hematopoietic cell transplantation for sickle cell disease. *Biol Blood Marrow Transplant*. 2018;24(2):219-227.
25. Stallings AM, Majhail NS, Nowacki AS, et al. Parent and guardian knowledge of hematopoietic cell transplantation as a treatment option for sickle cell disease. *J Pediatr Hematol Oncol*. 2019;41(3):187-193.
26. Bakshi N, Katoch D, Sinha CB, et al. Assessment of patient and caregiver attitudes and approaches to decision-making regarding bone marrow transplant for sickle cell disease: a qualitative study. *JAMA Netw Open*. 2020;3(5):e206742.
27. Khemani K, Ross D, Sinha C, Haight A, Bakshi N, Krishnamurti L. Experiences and decision making in hematopoietic stem cell transplant in sickle cell disease: patients' and caregivers' perspectives. *Biol Blood Marrow Transplant*. 2018;24(5):1041-1048.
28. Porter J. Beyond transfusion therapy: new therapies in thalassemia including drugs, alternate donor transplant, and gene therapy. *Hematology Am Soc Hematol Educ Program*. 2018;2018(1):361-370.
29. McCabe R, Pavlickova H, Xanthopoulou P, Bass NJ, Livingston G, Dooley J. Patient and companion shared decision making and satisfaction with decisions about starting cholinesterase medication at dementia diagnosis. *Age Ageing*. 2019;48(5):711-718.
30. Mathijssen EGE, Vriezekolk JE, Popa CD, van den Bemt BJF. Shared decision making in routine clinical care of patients with rheumatoid arthritis: an assessment of audio-recorded consultations. *Ann Rheum Dis*. 2020;79(2):170-175.
31. Gionfriddo MR, Branda ME, Fernandez C, et al. Comparison of audio vs. audio + video for the rating of shared decision making in oncology using the observer OPTION(5) instrument: an exploratory analysis. *BMC Health Serv Res*. 2018;18(1):522.
32. Williams D, Edwards A, Wood F, et al. Ability of observer and self-report measures to capture shared decision-making in clinical practice in the UK: a mixed-methods study. *BMJ Open*. 2019;9(8):e029485.
33. Hale KL, Wallace DD, Blanco-Duran D, et al. Conversations between Latina mothers and their child's mental health provider: an observational study of shared decision-making regarding pediatric patient mental health needs. *Patient Educ Couns*. 2020;103(1):96-102.
34. Muscat DM, Shepherd HL, Hay L, et al. Discussions about evidence and preferences in real-life general practice consultations with older patients. *Patient Educ Couns*. 2019;102(5):879-887.
35. Vortel MA, Adam S, Port-Thompson AV, Friedman JM, Grande SW, Birch PH. Comparing the ability of OPTION(12) and OPTION(5) to assess shared decision-making in genetic counselling. *Patient Educ Couns*. 2016;99(10):1717-1723.
36. Couët N, Desroches S, Robitaille H, et al. Assessments of the extent to which health-care providers involve patients in decision making: a systematic review of studies using the OPTION instrument. *Health Expect*. 2015;18(4):542-561.
37. Bakshi N, Sinha CB, Ross D, Khemani K, Loewenstein G, Krishnamurti L. Proponent or collaborative: physician perspectives and approaches to disease modifying therapies in sickle cell disease. *PLoS One*. 2017;12(7):e0178413.
38. Brand PL, Stiggelbout AM. Effective follow-up consultations: the importance of patient-centered communication and shared decision making. *Paediatr Respir Rev*. 2013;14(4):224-228.
39. Stiggelbout AM, Van der Weijden T, De Wit MP, et al. Shared decision making: really putting patients at the centre of healthcare. *BMJ*. 2012;344:e256.
40. Oerlemans AJM, Knippenberg ML, Olthuis GJ. Learning shared decision-making in clinical practice. *Patient Educ Couns*. 2020. https://doi.org/10.1016/j.pec.2020.09.034
41. Pieterse AH, Stiggelbout AM, Montori VM. Shared decision making and the importance of time. *JAMA*. 2019;322(1):25-26.
42. Den Hertog R, Niessen T. The role of patient preferences in nursing decision-making in evidence-based practice: excellent nurses' communication tools. *J Adv Nurs*. 2019;75(9):1987-1995.

How to cite this article: Mekelenkamp H, Smiers F, Camp N, Stubenrouch F, Lankester A, de Vries M. Decision making for hematopoietic stem cell transplantation in pediatric, adolescent, and young adult patients with a hemoglobinopathy—Shared or not? *Pediatr Blood Cancer*. 2021;68:e29090. https://doi.org/10.1002/pbc.29099