Parents' Experiences of Information and Decision Making in the Care of Their Child With Severe Spinal Muscular Atrophy

Beernaert, Kim; Lövgren, Malin; Jeppesen, Jørgen; Werlauff, Ulla; Rahbek, Jes; Sejersen, Thomas; Kreicbergs, Ulrika

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Parents' experiences of information and decision-making in the care of their child with severe spinal muscular atrophy: A population survey

Running head: Parent's care experiences for their child with SMA

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Kim Beernaert[a§], PhD; Malin Lövgren[b,c], PhD; Jørgen Jeppesen[d], PhD; Ulla Werlauff[d], PhD; Jes Rahbek[d], Msc; Thomas Sejersen[e], PhD; Ulrika Kreicbergs[b,c], PhD

[a]End-of-Life Care Research Group, Ghent University & Vrije Universtiteit Brussel (VUB), Ghent, Belgium

[b]Ersta Sköndal Bräcke University College, Department of Health Care Sciences, Palliative Research Centre, Box 11189, 100 61 Stockholm, Sweden.

[c]The Department of Women's and Children's Health, Paediatric Oncology and Haematology, Childhood Cancer Research Unit, Karolinska Institutet, Karolinska University Hospital, 177 77 Stockholm, Sweden.

[d]The National Rehabilitation Centre for Neuromuscular Diseases, Aarhus, Denmark.

[e]The Department of Women's and Children's Health, Paediatric Neurology, Karolinska Institutet, Karolinska University Hospital, Astrid Lindgren Children's Hospital, 177 77 Stockholm, Sweden.

§Corresponding author. Email address: kim.beernaert@ugent.be, Phone: +3293320795, Postal address: Ghent University Hospital, Building K, 6K3, Corneel Heymanslaan 10, 9000 Ghent, Belgium

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Abstract

Objective: This study aims to assess the experiences and wishes of parents of children with severe SMA regarding information and decision-making throughout the course of the illness.

Study design: A full population survey, conducted in 2015, among parents of children with severe SMA who were born in Denmark between January 1, 2003 and December 31, 2013. We used a study-specific questionnaire with items about experiences and wishes concerning the provision of information about diagnosis, treatment and end-of-life care.

Results: Among the 47 parents that were identified, 34 parents of 21 children participated. Eleven of them were non-bereaved and 23 were bereaved parents. All parents stated that healthcare staff did not take any decisions without informing them. A proportion of parents indicated that they were not informed about what SMA entails (32%), possible treatment options (18%), or the fact that their child would have a short life (26%) or that death was imminent (57%). Most of the bereaved parents who had wishes concerning how and where their child would pass away had their wishes fulfilled.

Conclusions: The study showed that healthcare staff did not take treatment decisions without parents being informed. However, there is room for improvement concerning information about what SMA entails, treatment options and prognosis. Possibilities of palliative care and advance care planning should be investigated for these parents, their child and healthcare staff.

Introduction

As treatment options for children with spinal muscular atrophy (SMA) increases, the choices and decisions that need to be made by healthcare staff and parents caring for them increase as well. Therefore it is more important than ever to learn about the experiences and wishes concerning information and treatment decisions of those caring for children with SMA to improve the quality of care. SMA shows a wide range of clinical severity, with type I and II resulting in shortened life expectation. Although there is no cure for any type of SMA to date, important developments have occurred in SMA medicine in recent years. A new drug, Nusinersen (Spinraza™), is the first drug treatment that has proved its ability to slow the progress of muscular atrophy, prolong life and improve muscle function in SMA type I and II[1,2]. However, there are practical challenges in delivering the drug and the medication is costly. At the same time, promising data from early-stage clinical trials of SMN1 gene therapy have indicated that additional therapeutic options are likely to emerge for patients with SMA in the near future[3]. Besides these drug evolutions, an updated clinical care guideline has been published, the Consensus Statement for Standard of Care in SMA (2017), which addresses pulmonary management and acute care issues as well as ethical issues and the choice of palliative care[1,4].

Because SMA is a rare disease, healthcare staff and parents often need to make difficult ethical decisions about the wellbeing or survival of the child, especially in severe SMA, despite a lack of experience and exposure[1]. Information, communication and planning are therefore indispensable to facilitate decision-making[5]. Only few studies have been conducted on parents' experiences of the care of children with severe SMA. To our knowledge, most of these are qualitative studies of very small samples focusing on the impact on the parent of living with a child with severe SMA and on what care was provided [6–8]. In a Swedish population-based study, conducted by authors of this manuscript, parents' experiences of the care of their child with severe SMA were explored [7,9,10]. In this population-based study, we explore the experiences and wishes of parents of children with severe SMA in Denmark regarding information and decision-making throughout the course of the illness.

Methods

Study design and study population

This Danish population survey was conducted in 2015 among parents who have or have lost a child with SMA. The participants are further referred to as non-bereaved and bereaved parents. The study population were all parents of children registered in the National Patient Register and/or the Cause of Death Register and/or the National Rehabilitation Center for Neuromuscular Diseases with the diagnosis SMA type I or II, born and diagnosed between January 1, 2003 and December 31, 2013. Parents' names and addresses were obtained through the Civil Registration System. To be included in the study, the parent needed to have or have had a child diagnosed with SMA type I or II (severe SMA), for whom respiratory support was considered as a treatment by healthcare professionals during the first year of life.

Data collection

Eligible parents were sent an invitation letter describing the study. Ten days later the study interviewer called the parents. If the parents agreed to participate, the questionnaire and an informed consent form were sent to each parent separately. Parents who did not answer the questionnaire within one month were sent a reminder.

Questionnaire development

The study-specific questionnaires, one for non-bereaved and one for bereaved parents, are based on Swedish study-specific questionnaires that include items about parents' experience of the care of their child with severe SMA [9]. The development of the questionnaire followed routines established by Charlton and others[11–13]. First, the questionnaires in Swedish were translated into Danish (UW and JJ). Then the Danish translations were validated in cooperation with a native speaker of Swedish with good knowledge of both Danish and SMA.

The final questionnaire for non-bereaved parents and bereaved parents included 59 and 75 questions respectively, of which 20 were considered for this study.

Measures

Both questionnaires (for non-bereaved and bereaved parents) consisted of questions regarding information given to family about diagnosis and treatment options and about how parents perceived their role in decision-making. The questionnaire for bereaved parents consisted of additional questions about the wishes and experiences concerning the child's end-of-life care. All questions used for this manuscript were closed-ended.

Statistical analysis

All data were entered into SPSS (version 24). Descriptive statistics were used to examine the parents' perception of their child's care. Despite low numbers of participants, we chose to present percentages for the reader's convenience.

Anonymity and ethical considerations

Permission to identify the parents was obtained from the Danish Health Authority (DHA). The DHA also approved the procedure for contacting the parents, including the phrasing of the contact letter. Permission to register data was obtained from The Danish Data Protection Agency.

Results

Forty-seven parents of 24 children were identified (Figure 1). In total, 34 parents of 21 children (11 non-bereaved parents of 8 children and 23 bereaved parents of 13 children) participated in the survey (response rate: 34/43, 79%). The parents' mean age was 38 and 40 for non-bereaved and bereaved parents respectively (Table 1). All children but two had SMA type II in the non-bereaved parent group and all but two had SMA type I in the bereaved group. The mean age of the child at the time of survey or death was 6 years and 17 months respectively. Other characteristics of the participating parents can be found in Table 1.

Parents' perception and experiences of the information provided about diagnosis and treatment decisions and their role in the decision-making

As shown in Table 2, 11 of the 34 parents (32%) stated that the information given did not help them understand what SMA entails. More than 80% of the parents (9/11 non-bereaved parents and 19/23 bereaved parents) indicated that they received information about treatment options. In these cases, five out of 11 (46%) non-bereaved parents indicated that they had only been informed about respiratory aid by tube in airway, whereas five out of 23 bereaved parents (26%) were only informed about ventilator by mask. Another five out of 23 bereaved parents (26%) did receive information about three options: respiratory aid by tube in airway, tracheostomy and ventilator by mask.

All parents stated that they had been informed about the treatment decisions that were made, although four of them said they had not participated in the decision-making. Nine non-bereaved parents (82%) and 18 (78%) bereaved parents felt confident about the decisions made.

Bereaved parents' experiences and wishes concerning their child's end-of-life care

Sixteen of the 23 parents (70%) stated that around the time of diagnosis they were informed that their child would have a short life (table 3). Thirteen (57%) stated the staff did not tell them in the days preceding of their child's death that that their child would pass away shortly.

Seventeen bereaved parents (74%) had wishes concerning where their child would pass away: ten (44%) wanted the death to take place at home and this wish was fulfilled for nine of them; six (26%) wanted their child to die in a children's hospital and this happened in five cases. For those who did not have wishes (n=6), four died in the intensive care unit, one in the children's hospital and one at home. A majority of bereaved parents (n=14) had wishes concerning *how* their child would pass away and 13 of them discussed this with the physician, and for all of them these wishes where fulfilled, although in four cases it did not proceed as planned. Of the 13 parents who talked about their wishes with the physician, for 11 of them the child died the way they wanted (not in table).

Discussion

The results of this nationwide study of non-bereaved and bereaved parents of children with severe SMA showed that healthcare staff did not take any treatment decisions without informing the parents first. Still, a fifth of the parents indicated that they were not informed about possible treatment options, and about a third said that they could not fully grasp the information about what SMA entails. One fourth of the bereaved parents indicated that they were not informed of the fact that their child would have a short life and more than half of them reported they did not receive information about their child's imminent death. Most of the bereaved parents who had wishes concerning how and where their child would pass away had these wishes fulfilled.

Strengths, limitations and considerations of the study

A strength of this study is that it covers the parent population of severe SMA children in Denmark during the years 2003 to 2013 well (79% response rate). Although it is a full population study, we report low numbers because SMA is a rare disease. In line with this, no statistical tests were possible due to the small sample size. Another strength of the study is the inclusion of both non-bereaved and bereaved parents. Differences found between non-bereaved and bereaved parents could also indicate differences in the care for SMA type I (most common in the bereaved group) or SMA type II (most common in the non-bereaved group). One has to take into account that the bereaved parents answer retrospectively, not only in terms of time but also in terms of loss. This may constitute a difference between the two groups. For ethical reasons it was impossible to link the parents with the children or with each other. The results should thus be interpreted with the knowledge that two parents from the same family may represent one child.

Interpretation of the results

A positive finding from this study is that all parents stated they were informed about decision-making regarding the treatment for their child and most of them indicated that they had participated in decision making. Moreover, about 80% of parents were confident about the decisions that were made (5 out of 34 parents were not). Similar results were found in a similar population-based Swedish

study[9,10]. These findings support the hypothesis that information given about treatment for the majority is in accordance with recommendations stated in the Consensus Statement for Standard of Care in SMA[1]. However, one fifth of parents stated that they did not receive information about possible medical treatment options, and a majority of parents who were given information stated that they had only received it for one treatment option, indicating that improved information is still needed.

One third of the parents indicated that they had not received information that helped them to understand what SMA entails. Most bereaved parents realized that their child would have a short life (70% – we have no information on the ones that were non-bereaved), but more than half of the parents stated they did not know when death was imminent (57%). In the Swedish study, 90% of bereaved parents said they knew that their child would have a short life and 73% of them were not told that death was imminent[10]. Not understanding the diagnosis and prognosis of your child might influence parents' decisions and wishes and makes it difficult to plan care. Predicting prognosis and imminent death in SMA is difficult.

Ten out of the 17 parents who stated that they had a wish concerning place of death stated home as the preferable place. Nine out of the ten did die at home. This is different from the study in Sweden, where fewer parents wanted their child to die at home, and where most children died in the children's hospital. However, a study of the place of death of children in 11 countries showed that Sweden has an average to high rate of home deaths for children with neuromuscular diseases compared to other countries (Denmark was not included in this study)[14]. This suggests that Denmark would have a high rate of home deaths in children with neuromuscular diseases, such as SMA, compared to other countries. Previous studies have shown that there is no clear evidence about the preference for place of death for children[15,16]. However, our study showed that over 80% of the children died where their parents wanted them to, in cases where they had a wish. This is higher than in the Sweden study (66%) and higher than the results that were found in another study in England by Beringer and Heckford (56%)[17]. Out of the seven parents who had no wish, four of them had a child who passed

away in the ICU. However, we do not have information on the quality of the end-of-life care delivered at the ICU, either at home or at the hospital.

Conclusion and recommendations

This population study including both bereaved and non-bereaved parents of children with SMA type I and II showed that healthcare staff did not take treatment decisions without parents being informed. Despite this, there are still some points where improvement is possible in terms of providing information about the diagnosis of SMA and what SMA is, treatment options and prognosis.

The introduction of new drugs such as Nusinersen that prolongs life and alleviates symptoms might imply that parents will be confronted with even more dilemmas regarding choices. Independently of the choice to prolong life or not, similar topics we investigated in this study will need to be considered by healthcare staff and parents. The options of palliative care [18], including advance care planning [19,20], might benefit these parents - and their child - by facilitating communication between all involved parties about diagnosis, prognosis, future treatment and (end-of-life) decision making[21].

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Guarantor. KB, UW and UK have full access to all the data in the study and take responsibility for the integrity of the data and the accuracy of the data analysis.

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Figure 1. Flowchart of the selection of study participants

Table 1. Demographics and clinical characteristics of the parents and their children, No.

	Non-bereaved parents N=11	Bereaved parents N=23
Gender of the parent		
Female	6	12
Male	5	11
Age of the parent		
Mean (SD)	37.6 (6.5)	39.7 (5.2)
Min-max	28-49	30-49
Levels of education		
Elementary school	1	4
Senior high school	3	5
University	7	14
Place of residence		
Rural	4	7
Small town (10,000-50,000)	5	8
Medium-sized city (50,000-100,000)	0	1
In a big city (100,000 inhabitants or more)	2	6
The child's age (months) at diagnosis		
Mean (SD)	10.5 (7.0)	4.0 (3.5)
Min-max	0-20	0-15
Child's age at time of follow-up/death		
Mean (SD)	5.7 (2.6) years	17 (31) months
Min-max	2-10 years	0-116 months
The child's diagnosis		
SMA type I	2	19
SMA type II	9	1
SMA type I or II	0	1

Table 2. Parents' perception and experiences of the provision of information about diagnosis and treatment decisions, No. (%)

	Non-bereaved parents, N= 11	**Bereaved parents, N= 23**
Were you given information that helped you understand what spinal muscular atrophy is? *		
Yes	7 (64)	15 (68)
No	4 (36)	7 (32)
Have you been given information about possible medical treatment options for your child?		
Yes	9 (82)	19 (83)
No	2 (18)	4 (17)
If special respiratory support has been discussed, what options were you informed about?		
No information	2 (18)	4 (21)
Respiratory aid by tube in airway	5 (46)	2 (11)
Ventilator by mask	2 (18)	5 (26)
Respiratory aid by tube in airway and ventilator by mask	0 (0)	1 (5)
Respiratory aid by tube in airway and tracheostomy	0 (0)	2 (11)
Tracheostomy and ventilator by mask	0 (0)	1 (5)
Respiratory aid by tube in airway and tracheostomy and ventilator by mask	2 (18)	5 (26)
How have you perceived decisions made regarding the treatment of your child?*		
Informed, and participated in decision	8 (73)	17 (81)
Informed, but did not participate in decision	3 (27)	1 (5)
Not informed, healthcare staff made decision	0 (0)	0 (0)
Not applicable, no decisions have been taken	1 (9)	3 (14)
Have you felt confident about the decisions made regarding the treatment of your child?*		
Yes	9 (82)	18 (78)
No	2 (18)	3 (13)
Not applicable, no decisions have been made	1 (9)	2 (9)

*The numbers may not equal total N as parents may have ticked more than one response alternative or did not ticked a response (missing).

Table 3. Bereaved parents' experiences and wishes concerning their child's end-of-life care, No. (%)

	Bereaved parents, N=23
In connection with the diagnosis, were you informed that your child in all likelihood would have a short life?	
Yes	16 (70)
No	6 (26)
Not answered	1 (4)
In your opinion, when you were told that your child in all likelihood would have a short life, was it communicated in as considerate a manner as possible?*	
Yes	18 (78)
No	4 (17)
I was never told	2 (9)
Did any member of the staff tell you, in the days preceding your child's passing, that your child would pass away shortly?	
Yes	10 (44)
No	13 (57)
Did you, as a parent, have any wishes concerning <u>where</u> your child would pass away?	
Yes, at home	10 (44)
Yes, in a children's hospital	6 (26)
Yes, in another place	1 (4)
No	6 (26)
Where did your child pass away?	
At home	10 (44)
In the general pediatric unit	8 (35)
In the intensive care unit	5 (22)
In the emergency care unit	0
In another place	0
Were your wishes about location of death fulfilled?	
Yes, at home	9 (39)
Yes, at the children's hospital	5 (22)
No, instead of home, they died in the children's hospital	1 (4)
No, instead of children's hospital, they died in the ICU	1 (4)
There were no wishes, they died at home	1 (4)
There were no wishes, they died in the children's hospital	2 (9)
There were no wishes, they died in the ICU	4 (17)
Did you, as a parent, have any wishes concerning <u>how</u> your child would pass away?	
Yes	14 (61)
No	9 (39)
Did you talk to your child's physician about your wishes?	
Yes	13 (57)
No	10 (44)
Were these wishes fulfilled?	
Yes, it was as I had wanted and planned for	9 (39)
Yes, it was as I had wanted, but different than what I had planned	4 (17)
No	1 (4)
Not applicable – I had no particular wishes	7 (30)

*More than one response alternative could be chosen.

References

1. Finkel RS, Mercuri E, Meyer OH, et al. Diagnosis and management of spinal muscular atrophy: Part 2: Pulmonary and acute care; medications, supplements and immunizations; other organ systems; and ethics. *Neuromuscul Disord*. November 2017.

2. Finkel RS, Mercuri E, Darras BT, et al. Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy. *N Engl J Med*. 2017;377(18):1723-1732.

3. Groen EJN, Talbot K, Gillingwater TH. Advances in therapy for spinal muscular atrophy: promises and challenges. *Nat Rev Neurol*. 2018;14(4):214-224.

4. Wang CH, Finkel RS, Bertini ES, et al. Consensus Statement for Standard of Care in Spinal Muscular Atrophy. *J Child Neurol*. 2007;22(8):1027-1049.

5. Bluebond-Langner M, Hargrave D, Henderson EM, Langner R. "I have to live with the decisions I make": laying a foundation for decision making for children with life-limiting conditions and life-threatening illnesses. *Arch Dis Child*. 2017;102(5):468-471.

6. Higgs EJ, McClaren BJ, Sahhar MA, Ryan MM, Forbes R. "A short time but a lovely little short time": Bereaved parents' experiences of having a child with spinal muscular atrophy type 1. *J Paediatr Child Health*. 2016;52(1):40-46.

7. Hjorth E, Kreicbergs U, Sejersen T, Lövgren M. Parents' advice to healthcare professionals working with children who have spinal muscular atrophy. *Eur J Paediatr Neurol*. 2018;22(1):128-134.

8. von Gontard A, Rudnik-Schöneborn S, Zerres K. Stress and Coping in Parents of Children and Adolescents with Spinal Muscular Atrophy. *Klin Pädiatrie*. 2012;224(4):247-251.

9. Lövgren M, Sejersen T, Kreicbergs U. Information and treatment decisions in severe spinal muscular atrophy: A parental follow-up. *Eur J Paediatr Neurol*. 2016;20(6):830-838.

10. Lövgren M, Sejersen T, Kreicbergs U. Parents' Experiences and Wishes at End of Life in Children with Spinal Muscular Atrophy Types I and II. *J Pediatr*. 2016;175:201-205.

11. Charlton R. Research: is an "ideal" questionnaire possible? *Int J Clin Pract*. 54(6):356-359. http://www.ncbi.nlm.nih.gov/pubmed/11092107.

12. Kreicbergs U, Valdimarsdóttir U, Onelöv E, Henter J-I, Steineck G. Talking about Death with Children Who Have Severe Malignant Disease. *N Engl J Med*. 2004;351(12):1175-1186.

13. Grenklo TB, Kreicbergs UC, Valdimarsdóttir UA, Nyberg T, Steineck G, Fürst CJ. Communication and trust in the care provided to a dying parent: a nationwide study of cancer-bereaved youths. *J Clin Oncol*. 2013;31(23):2886-2894.

14. Håkanson C, Öhlén J, Kreicbergs U, et al. Place of death of children with complex chronic conditions: cross-national study of 11 countries. *Eur J Pediatr*. 2017;176(3).

15. Bluebond-Langner M, Beecham E, Candy B, Langner R, Jones L. Preferred place of death for children and young people with life-limiting and life-threatening conditions: A systematic review of the literature and recommendations for future inquiry and policy. *Palliat Med*. 2013;27(8):705-713.

16. Bluebond-Langner M, Beecham E, Candy B, Langner R, Jones L. Problems with preference and place of death for children too. *BMJ*. 2015;351:h6123.

17. Beringer AJ, Heckford EJ. Was there a plan? End-of-life care for children with life-limiting conditions: a review of multi-service healthcare records. *Child Care Health Dev*. 2014;40(2):176-183.

18. Ho C, Straatman L. A review of pediatric palliative care service utilization in children with a progressive neuromuscular disease who died on a palliative care program. *J Child Neurol*. 2013;28(1):40-44.

19. Hauer JM, Wolfe J. Supportive and palliative care of children with metabolic and neurological diseases. *Curr Opin Support Palliat Care*. 2014;8(3):296-302.

20. Liberman DB, Song E, Radbill LM, Pham PK, Derrington SF. Early introduction of palliative care and advanced care planning for children with complex chronic medical conditions: a pilot study. *Child Care Health Dev*. March 2016.

21. Lykke C, Ekholm O, Schmiegelow K, Olsen M, Sjøgren P. All-cause mortality rates and home deaths decreased in children with life-limiting diagnoses in Denmark between 1994 and 2014. *Acta Paediatr*. March 2018.