Parents' perspectives on nusinersen treatment for children with spinal muscular atrophy

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ABBREVIATIONS
SMA Spinal muscular atrophy

AIM To gain insight into parents' perspectives about their decision-making process concerning nusinersen treatment for their child, including perceived needs and concerns, and to explore factors that influence this process.
METHOD This was an exploratory qualitative interview study among parents of children with spinal muscular atrophy types 1 to 3. Data were analysed using inductive thematic analysis.
RESULTS Nineteen parents of 16 children representing 13 families participated. A wide variety of perspectives was reported ranging from a biomedical approach, which focused on battling the disease, to a holistic approach, which aimed for a good quality of life for their child. The most important factors that helped parents to decide were honest and neutral communication with their physician and access to available information.
INTERPRETATION It is important physicians understand that there are different perspectives influencing the decision-making process. Physicians should create an environment that allows parents to accept or reject treatment by communicating honestly and openly with them and by discussing both options extensively. Clear information about pros and cons, recent developments in research, and the experiences of other parents should be made available to enable parents to make an informed decision.

Spinal muscular atrophy (SMA) is a severe neuromuscular disorder caused by homozygous loss of function of the survival motor neuron 1, telomeric (*SMN1*) gene.[1] The disease is characterized by disrupted gross motor development during infancy or early childhood, severe medical complications, such as problems with feeding, airway clearance, and breathing, and scoliosis.[2–4] There is considerable variation in severity, reflected by the distinction of types 1 to 4, based on age at onset and acquired motor milestones. Within the spectrum of the disease, patients with SMA range from very weak infants to ambulant children and adults. In early-onset SMA (type 1), symptoms of the disease appear very early in life (before the age of 6mo), progression is rapid, and life expectancy was reduced to 1 to 2 years before treatment options became available.[5]

In December 2016, the first disease-modifying therapy (the antisense oligonucleotide, nusinersen) became available and was eligible for reimbursement in the USA, followed by the European Union in May 2017.[6] Nusinersen is administered via repeated intrathecal injections; it has been shown to stimulate physical improvements and prolong life but cannot cure SMA.[5,7–10] Parents caring for a child with SMA are confronted with many uncertainties and concerns regarding the prognosis and future of their child. At the same time, they have to decide whether or not to opt for nusinersen treatment.[11,12] Their beliefs regarding the health threats caused by SMA influences their perception of the need for this treatment and their concerns about it;[13–15] they will need to weigh up these beliefs before deciding whether or not to commence treatment.

Recently, Pacione et al.[16] described parental concerns about nusinersen. Parents' main concerns focused on financial costs, the lack of empirical data on the positive effects, and the side effects of nusinersen.[16] However, only three parents who decided against nusinersen treatment were included in the study.

To support parents in their decision-making process, it is important to understand their perceptions and expectations of this relatively new medical treatment.[17] Therefore, the

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objective of this study was to gain an insight into parents' perspectives on the need for and concerns about nusinersen treatment that underlay their decision-making concerning nusinersen treatment for their child, and to explore factors that influenced their decision-making process.

METHOD

An exploratory qualitative interview study was conducted. Data were analysed using inductive thematic analysis[18,19] and reported in accordance with the guidelines of the Standards for Reporting Qualitative Research checklist.[20]

This was part of a larger study that aimed to gain an insight into parents' perspectives in caring for a child with SMA.[12] At the time of data collection, nusinersen was reimbursed in the Netherlands for children with SMA aged 9 years 6 months or younger.[21] Nusinersen was implemented by the national centre of expertise for SMA, which performed a screening to identify children eligible for nusinersen treatment. Parents were presented with the option of whether or not their child would receive the treatment.

Sample

The Dutch national SMA database, which includes the majority of Dutch patients diagnosed with SMA,[2] was used to invite parents based on the following criteria: child with SMA aged 9 years 6 months or younger with types 1 to 3 SMA who were offered nusinersen treatment. Those who were not fluent in Dutch or English were excluded.

Recruitment was performed by the day-to-day curator of the Dutch national SMA database (FA) based at the University Medical Center Utrecht, who identified eligible parents in the database. Eligible parents (n=45 families) were informed about this study and invited to participate by letter. If parents wanted to participate in this study, they were asked to give their permission to be contacted by e-mail. Subsequently, they were approached by the researchers for an interview.

Maximum variation in the spectrum of considerations and decisions was checked by including both parents who accepted, as well as those who declined nusinersen treatment.[22]

The medical ethics committee of the University Medical Center classified this study as exempt from the Medical Research Involving Human Subjects Act (17-904). All participants gave written informed consent.

Data collection

Data collection consisted of a single semi-structured, face-to-face interview (by MF and CS). Before the study, a topic list based on the literature and research team's expert knowledge was developed (Appendix S1, online supporting information). We aimed to include both parents in one interview to get a broad perspective on the considerations and decision-making process.

Before the interview, participants were given the choice of location to facilitate a setting where they would feel most comfortable when describing their experiences.

What this paper adds

- Parents perceived different needs and concerns about nusinersen treatment, which emphasized individual differences.
- Parents' perspectives varied from battling the disease to preserving quality of life.
- Life expectancy, stopping deterioration, and improving quality of life were the perceived benefits of nusinersen treatment.
- Open communication about the pros and cons of treatment with clinicians facilitated decision-making.
- Clear and honest information facilitated the alignment of values and goals.

Interviews were audio-recorded and transcribed verbatim. We included all parents who were willing to participate in this study and felt we reached code saturation.[23]

Data analysis

The process of data collection and data analysis was iterative. A thematic analysis was conducted as described by Braun and Clarke.[19] The six phases of this method and the contribution of the members of the research team are presented in Table 1. Throughout the study, the research team (MVK, MCK, CS, MF) familiarized themselves with the data, contributed to the identification and development of codes and (initial) themes, and discussed the interpretation of (sub)themes. We continuously integrated and discussed themes from preliminary codes to final results.

To ensure the quality of the data and correct application of the six phases of the thematic analysis, Braun and Clarke[19] organized the criteria for each phase in a 15-item checklist. All 15 items were met by this study. This included criteria such as 'themes have been checked against each other and back to the original data set' or 'themes are internally coherent, consistent, and distinctive'.[19] For a full overview of the checklist, see Appendix S2 (online supporting information). The MAXQDA software was used to support data analysis (VERBI Software, Berlin, Germany).

RESULTS

Participants

In total, 13 interviews were conducted with 19 parents of 16 children representing 13 families (response rate: 28.9%; Table 2). In six families, both parents participated. Of the remaining seven families, only the mother participated. An overview of the inclusion of participants is provided in Figure S1 (online supporting information).

The parents of two families declined nusinersen treatment for their child. Both children died before the interview was conducted. Three of the 13 families had two or more children diagnosed with SMA. In two of these three families, only one child (in each family) was offered treatment. In one family, the youngest child had died before nusinersen was available. The interviews were conducted at the preferred location of the parent(s) between January and November 2018 and lasted 60 to 90 minutes.

Parents' perspectives on nusinersen

The parents' main focus was to provide a good life for their child by trying to manage the physical symptoms of

Table 1: The six phases of thematic analysis[19]

Phase	Description
1. Familiarize yourself with the data	The interviews were transcribed (MVK, CS) verbatim. The accuracy of the transcriptions was checked. Transcripts were read and reread by five authors (CS, MCK, MF, MV, MVK) and the notes of the initial ideas were collected and discussed within the research team. Adjustments of the interview guide were discussed (MF, CS, MCK) and implemented.
2. Generate initial codes	First, two transcripts were open-coded by five authors (MVK, CS, MCK, MF, MV) independently. The findings and codes were compared and discussed until consensus was reached. Next, three interviews were coded and discussed by three authors (MVK, CS, MCK) resulting in a preliminary code tree. Finally, 10 transcripts were coded by CS and MVK. These transcripts and codes were compared and discussed during meetings with a qualitative research expert (MCK) until consensus about the design interpretation of the codebook and data was reached.
3. Search for themes	Based on the list of codes, three authors (MVK, CS, MCK) searched for and discussed potential themes and interrelated elements. Potential descriptions of the (sub)themes were formed by MVK and discussed with CS and MCK until consensus was reached.
4. Review the themes	The potential descriptions of the themes were checked by the first author (MVK) with the original transcripts and codes. Inconsistencies were discussed in joint meetings (MVK, CS, MCK). Refinements of the description of the themes were made (MVK, CS).
5. Define and name the themes	The first author (MVK) worked out the specific thematic content then, with CS, worked out the overall story line. The preliminary results were presented to WLP, an expert in SMA childcare. Revised themes were presented in an expert meeting that included health professionals working with children with SMA and their parents and the research team (IC, MK, MCK, AVM); refinements were made.
6. Produce a report	CS and MVK wrote a first draft of the scientific report and selected relevant quotes to illustrate the themes. The last author reviewed the report and necessary adjustments were made. The report was submitted to the research team (all authors) for critical assessment. Feedback was processed and refinements in the manuscript were made until consensus was reached.

Table 2: Characteristics of the parents and their children

Characteristic	*n* (%)
Parents	19 (100)
Sex of parent	
Male	6 (31.6)
Female	13 (68.4)
Age of parent, y	
<29	1 (5.3)
30–39	12 (63.2)
40–49	2 (10.5)
>50	2 (10.5)
Unknown	2 (10.5)
Education	
Low	8 (42.1)
Middle	8 (42.1)
High	3 (15.8)
Children	16 (100)
SMA type	
1	7 (43.8)
2	5 (31.3)
3	3 (18.8)
Diagnosed with SMA, not yet specified[a]	1 (6.3)
Age of child at interview, y	13 (81.3)
0–1	3 (18.8)
2–3	3 (18.8)
4–5	4 (25)
6–8	3 (18.8)
Deceased: age of child at death, mo	3 (18.8)
0–4	2 (12.5)
5–6	1 (6.3)
Families	13 (100)
Number of families with typically developing siblings	10 (76.9)
Number of families with siblings with SMA	3 (23.1)

[a]Child was too young to be able to specify SMA type. SMA, spinal muscular atrophy.

SMA. Many parents wanted to offer their child a chance of managing the disease, thus providing them the opportunity of a good life. In addition, they emphasized that they would do everything to keep their child with them; nusinersen treatment offered them this opportunity.

Rejecting the treatment, while knowing that deterioration would progress, felt like giving up on their child or allowing deterioration: 'You know what will happen if you don't do anything … it will get worse and he will soon end up in a wheelchair or worse' (mother of child with type 3 SMA). Therefore, most parents stated that refusing treatment did not feel like a choice for them: 'I more or less took it for granted: yes, surely he should be given it?' (mother of child with type 3 SMA). A few parents of children from the minority of the SMA population eligible for treatment reported they felt privileged that their child was granted this opportunity: 'It's there for the taking. But we are so happy and are very aware that we belong to a very small group who are eligible for this' (father of child with type 2 SMA).

Some parents mentioned that they had been very eager to get nusinersen treatment for their child, even before it was available in the Netherlands. Therefore, they had kept a close eye on the developments about nusinersen. These parents reported, for instance, that they had actively tried to maximize their chances of getting access to nusinersen treatment by registering their child at the national centre of expertise for SMA. While many parents stated that their stance was obvious, given the chance of stabilization of the disease, others emphasized the importance of also weighing up the influence of nusinersen on their child's quality of life and considering the possible pros and cons in relation to the expected physical improvement and quality of life.

Perspectives on a spectrum: from a biomedical to a holistic approach

Based on the diversity in parental perspectives, we identified a spectrum with at one end a mainly biomedical approach expressed as 'battling the disease', and at the other, parents who held a broader, more holistic approach to their child's situation. The more 'biomedical' parents based their approach on the perceived needs, such as aiming to cure the disease. Parents with a 'holistic' approach predominantly aimed for a good quality of life for their child and emphasized that this included questioning and weighing their child's quality of life versus aiming for a cure at all costs. Parents varied in their perspectives and position within the spectrum at different time points. Therefore, the spectrum can be seen as a dynamic field.

Parents' perceived needs

Overall, parents reported the possible effect of nusinersen on life expectancy, stopping deterioration, and increasing independence as the most important needs. Parents with a predominantly biomedical perspective focused their considerations about treatment on their perceived needs and their importance, rather than their concerns.

Life expectancy

All parents reported that they hoped nusinersen would prolong their child's life. For many parents, this was the most important reason to aim for nusinersen treatment. Parents assessed nusinersen as being life-saving and a last resort:

> You're going to see your child deteriorate; more and more things will happen. You're going to see your child being able to do less and in the end … it's over. So, what do you want? To be able to postpone it.
>
> (Father of child with type 2 SMA)

For many parents, this was why deciding on whether to receive nusinersen treatment did not feel like a choice. Some parents adopting a mainly biomedical approach indicated that their concerns about complications or pain were outweighed by their hope of prolonging life.

Stopping deterioration and enabling independence

Many parents indicated that stopping deterioration or stabilizing the current physical state of their child was important for them. Furthermore, they hoped that treatment would result in physical improvement. Whereas parents of children with type 1 SMA mainly reported that they hoped to stop their child's deterioration, parents of children with types 2 or 3 SMA indicated that stabilizing the current situation might mean that their child could remain independent in the future: 'The most important thing is that my child stops deteriorating. Because the way my child is now, he/she can [still function] to some extent in the future' (father of child with type 2 SMA).

Most parents adopting a mainly holistic approach hypothesized that increased independence would improve their child's ability to live a 'normal life' and increase societal participation; as such, it would result in an enhanced quality of life. This would also mean fewer caregiving demands on their part.

Parents' perceived concerns

Parents reported concerns about the complications of treatment, quality of life of their child, availability of nusinersen and alternative medications, and possible increased and prolonged caregiving demands on them. Specifically, parents adopting a mainly holistic approach balanced their concerns about treatment against what they considered necessary.

Complications of treatment

Parents adopting a mainly holistic approach indicated that they worried about the possible treatment complications for their already vulnerable child:

> My child has to have an anaesthetic, it is a spinal tap. Maybe it will cause a lot of pain or it might actually make my child tired. If you don't think there will be any benefit, as in: progress or stability, then you have to ask yourself: 'what are you doing?'.
>
> (Mother of child with type 1 SMA)

Some parents worried about their child's ability to cope with the treatment physically; they reported being afraid that treatment would cause too much suffering and effort for their child. Therefore, their physician's assessment was very important to them.

Parents adopting a mainly biomedical perspective also reported that they thought the treatment might be too intensive for their child and that side effects might occur. However, these parents stated that the expected side effects were not life-threatening and as such acceptable compared to the greater good of a cure:

> If the risk had been 50% that our child would die or be given medication, then it would have been a completely different matter. Then we really would have had to think: are we going to give it or not. But the side effects were not so bad that I think: well then!
>
> (Mother of child with type 1 SMA)

Concerns about their child's quality of life

Some parents reported that they first considered what level of quality of life for their child was acceptable and whether treatment could add to the child's quality of life. Parents adopting a mainly holistic approach emphasized that quality of life for their child during and after treatment was the most important factor in making the final decision about nusinersen, for example, when considering issues of treatment intensity or perceived suffering for their child. Other parents reported being concerned that the intensity of treatment and possible side effects might intrude too much

in their child's daily living. However, they stated that this consideration was not a reason to reject the treatment but something to keep in mind and monitor.

In two cases, the parents of children with type 1 SMA rejected nusinersen treatment. Within the context of extending life, for these parents the decisive consideration was that, from their perspective, nusinersen treatment could not guarantee a sufficient level of quality of life for their child. They indicated that they did not want their child to suffer unnecessarily due to prolonging a poor quality of life.

> In my opinion it's a very selfish choice because the only thing you want to achieve is to keep your child with you. And then, you are putting your child through this. Well, I can't reconcile this.
>
> (Mother of child with type 1 SMA)

Another concern of parents adopting a mainly holistic approach was that the possible prolongation of life also increased the chance that their child would be more aware of their difficult situation and of being different from other children, which might create a greater sense of emotional pain.

> You should know our child has not been aware of the fact that it existed, which offers some peace [. . .] and maybe he has had less pain. And that we have not had to explain to him why we decided to give specific treatment.
>
> (Mother of child with type 1 SMA; child deceased)

Effects of nusinersen are uncertain

Many parents indicated that the actual effects of nusinersen treatment on their child are still unclear: 'We did think something like: oh God, just imagine! And quite quickly we said: but, you know, there are no guarantees' (mother of child with type 2 SMA). Overall, most parents stated that although the positive effects of nusinersen could not be guaranteed, they decided to give nusinersen treatment the benefit of the doubt. Specifically, parents of a child with type 2 or 3 SMA indicated that the perceived risks were not that high and the treatment, therefore, certainly seemed worth trying, given a reasonable quality of life.

Increased parental caregiving demands

Some parents mentioned that they worried about the risk that extending life by giving nusinersen would also increase and intensify the child's caregiving demands. In hindsight, a few parents felt unsure whether they would be able to fulfil the child's increased caregiving demands while maintaining a healthy mental and physical state themselves: 'And also for ourselves: we can't give 24/7 care. Really it was nearly killing us' (father of child with type 1 SMA). Parents adopting a mainly biomedical approach mentioned that they hoped the caregiving demands would decrease in the future due to the possible increased independence of their child. According to some parents, increased independence could mean that their child might do more things independently and consequently relieve the burden of parental caregiving: 'What if Spinraza [nusinersen] works [. . .]? If only our child would be able to turn over? Then of course it suddenly becomes a whole lot easier for us' (father of child with type 2 SMA).

What facilitated or hampered parents in their decision-making process?

During the interviews, parents gave an insight into what helped or hindered them in deciding on nusinersen treatment for their child. They reported the following factors: communication with their physician, availability of information, and unequal availability of nusinersen within one family.

Communication with their physician

All parents mentioned that the treating physician played a major role in facilitating their decision-making process. First, many parents stated that they regarded their treating physician as their main source of information. Their physician provided them with the most up-to-date information and was able to communicate this. Parents also reported that it helped if this information was communicated in a clear and understandable manner. Second, many parents reported that trust in the expertise of their physician facilitated their decision-making process. It was important to parents that the pros and cons of treatment were honestly and carefully presented to them by their physician.

Parents stated that trust in their physician was strengthened when they felt they had an equal and open conversation with their physician and when their physician listened to them carefully. In addition, parents indicated that if their physician was positive about the opportunity for improvements in the child, they were more likely to decide in favour of treatment: 'All this experience. I feel that physicians are very professional, open, and honest. So, I have confidence in them. If they say so, [. . .] we should do that' (mother of child with type 1 SMA).

Third, parents reported that (the feeling about) making their own choice was facilitated by the fact that physicians remained neutral about the final decision that they, as parents, had made. This neutral position made parents feel in control when making their decision.

> Mother: No, the physicians certainly did not put pressure on us. They involved us at every stage.
> Father: The physician was very reticent. [. . .] I appreciated that. I think that responsibility for making the decision should rest with the parents.
>
> (Mother and father of child with type 2 SMA).

Additionally, parents indicated that it was important for them that their decision, regardless of the choice made by them, was accepted by the physician. They mentioned that this helped them to communicate about their decision: 'Something the physician said I found really important: whichever choice you make, there is no wrong choice. [. . .]

You just felt you had support for any decision' (father of child with type 1 SMA).

Availability of information

In addition to the information provided by the physician, most parents stated that they searched for information on the Internet to get an idea of the effect of and experiences with nusinersen. A few parents reported that they searched for information in medical articles about trials and effects to obtain information about the possible effects of treatment with nusinersen. While some parents stated that they managed to 'struggle' through a medical article, others indicated that it was difficult for them to understand: 'It is important that people are well-informed about what is involved. And something that is a real problem [. . .] are all those difficult scientific papers. I can't concentrate on them. Some people can't even read English' (mother of child with type 2 SMA). Other parents reported searching for information about the experiences of other parents with nusinersen treatment. This information was mainly found on forums and in online videos. Parents indicated that online videos of children already receiving nusinersen were perceived as hopeful.

Searching for information was sometimes perceived as difficult because the information was scattered over the Internet. This was mentioned as an obstacle in making a well-informed decision about whether or not to start with nusinersen. Based on all available information, parents tried to form or adjust their expectations about treatment: 'They also mentioned a [video clip] that you can see, that shows the most fantastic result. There are also other children who may die. It is all – it is not all straightforward. If only' (mother of child with type 1 SMA).

Unequal availability of nusinersen within one family

In this study, three families had several children with SMA. Due to the procedure for making nusinersen available in the Netherlands, it was possible that one child from such a family was offered the medicine, whereas the other child was not eligible for treatment. A few parents indicated that this made them feel conflicted during and after the decision-making process:

> The only thing that bothered us is just finding it really difficult that – two sides of the story: on the one hand we are really happy that [our child] is getting Spinraza [nusinersen], but we also have someone at home who is not getting it yet and that means we sometimes think ... shouldn't we have waited for them to receive treatment together?
>
> (Mother of child with type 3 SMA).

DISCUSSION

In this study, we aimed to gain an insight into parents' perspectives on nusinersen treatment and identify factors that helped parents to make a treatment decision. A wide variety of perspectives was reported; we identified a spectrum with, at one end, a biomedical approach, which focused on battling the disease, and at the other, a mainly holistic approach, which focused on providing a good quality of life for the child. Based on the variety in perspectives, parents were flexible in their position within the spectrum at different time points. The most important factors that helped parents to decide were honest and neutral communication with their physician and easy access to scientific information and other parents' experiences about the treatment.

Parents with a predominantly biomedical perspective mainly reported a focus on the need for treatment. In previous end-of-life studies, parents coping with loss appeared to largely influence parents' decision-making.[24,25] Difficulty in coping with loss focused the parental perspective towards preserving the child's life and the parents' life with the child, concentrating on leaving no stone unturned to prolong life, whereas parents who could cope with loss acted and thought in the interest of the child's quality of life.[25] It is possible that fear of losing a child accentuates a biomedical perspective that strengthens a focus on battling the disease (at all costs). Similarly, Qian et al.[11] reported that parents opted for invasive medical treatment even though the treatment outcome was uncertain. In contrast, parents with a more holistically focused perspective reported both needs and concerns, including the child's perspective. This suggests that parents with this perspective had a wider range of concerns and needs and had more thoughts about the consequences of the disease for their child and their family in the long term. Many parents mentioned concerns about quality of life, functional status, uncertainty about the effectiveness of nusinersen, and side effects, which are in line with the concerns of parents in the study by Pacione et al.[16] Unlike Pacione et al.,[16] the cost of medication was not mentioned in this study as a barrier or concern of nusinersen treatment. We assume this is related to the Dutch health service where at the time of data collection, nusinersen was reimbursed in the Netherlands for children with SMA, aged 9 years 6 months or younger.

Pacione et al.[16] suggested that parents of children with types 2 and 3 SMA may approach treatment decisions differently from parents of children with type 1 SMA. In our study, we found that a few parents of a child with type 1 SMA were mainly focused on increasing life expectancy, representing a strong biomedical perspective. However, some parents with a child with type 1 SMA described that they shifted their focus from life preservation to letting go and creating a life worth living, according to a more holistic perspective.[25,26] It is possible that fast progression of the disease, as seen in children with type 1 SMA, can shift the parents' approach from biomedical to holistic. In addition, many parents with a child with types 2 and 3 SMA in our study reported that they wanted to fight the disease to stop deterioration and reported a perspective with a mainly biomedical focus. However, other parents with a child with types 2 and 3 SMA stated that stopping deterioration was

important because it increased independence, creating a better quality of life, which is similar to a holistic perspective.

Easy access to different kinds of information about the treatment, and open and non-judgemental communication with their physician, were perceived by the parents as important factors in creating a frame of reference. This helped parents to weigh up their options about whether or not to start nusinersen treatment. Previous studies in different areas have also shown the importance of the role and expertise of physicians when making a medical decision.[27,28] Information is important for parents to feel confident about the treatment decision they make.[25,29] However, for parents to feel in control of the decision-making process about the treatment of their child, providing information alone is not enough.[28] It is important that physicians empower parents by providing information in a neutral and honest manner.[17,26,30] Only then do all options in the decision-making process become available, including room to decline treatment.

We aimed to acquire a broad understanding of parents' perspectives on nusinersen treatment. A strength of our study is the inclusion of all types of SMA in children, and of parents who accepted or rejected nusinersen treatment. However, only parents of children with SMA type 1 declined treatment. In the registry, no families of children with type 2 SMA rejected nusinersen treatment.

A limitation of this study is the selective recruitment. Parents who participated in the study made their decision about nusinersen treatment before the interview; therefore, it was likely they would substantiate the choice they made. This means that these parents may have reasoned differently at the time of the interview than parents who are still in the decision-making process. In addition, we included parents who were fluent in English and Dutch. This means the sample represents only part of the Dutch and international cultural settings. Future research should focus on the perspectives of parents with different cultures and backgrounds and the experiences and perspectives of the children.

Finally, due to the nature of sample selection, parents who participated in this study may have been more motivated and prone to elaborate on their thoughts and opinions about nusinersen. This might suggest that the perspective of parents with less explicit ideas or feelings about nusinersen treatment might be less represented in this study. However, we felt we reached code saturation.

Implications for clinical practice

In this study, parents reported that their contact with their physician played an important role in their decision about whether or not they wanted their child to receive nusinersen treatment. Physicians can create an environment for parents that gives them room to say 'yes' or 'no' to treatment by communicating honestly and openly and by discussing both options (accepting or refusing treatment) extensively.[17,28] Discussing the detailed consequences of the different choices also helps to broaden the perspectives of parents[24,30] and provides an overview. This is particularly desirable for parents who mainly adopt a biomedical perspective and are inclined to focus on survival. Discussing all options might broaden their perspective on the situation, otherwise they might focus on one aspect in particular.

Easy access to reliable information, preferably from different sources, enabled parents to feel more confident in making their treatment decision. Clear information about the pros and cons, recent developments in research, and practical information about treatment can be made available, for example, through a website. In addition to the (often medical) information about the treatment, the experiences of other parents are important for obtaining a broad view of what is involved in nusinersen treatment.[17]

By providing scientific information, other parents' experiences, clear communication, and extensively discussing all treatment options including their pros and cons, health care professionals can empower parents and facilitate ownership of their decision-making. Providing empowerment and ownership of the decision-making process about a novel treatment can be extended to all parents with a child with a neurological disorder.

Conclusion

Parents reported a wide variety of views about nusinersen treatment, which varied across a spectrum from a biomedical to a holistic perspective on the situation of their child; this might vary at different points in time. Parents adopting a mainly biomedical perspective placed more focus on the need for treatment, such as increasing life expectancy, and had fewer concerns. Parents adopting a mainly holistic perspective focused on both the need for treatment but also on the concerns they had about it. They placed more emphasis on stopping the deterioration of their child and on improving or maintaining their child's quality of life. It is important to take these differences in parental perspectives into account when presenting a new treatment option.

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DATA AVAILABILITY STATEMENT

Research data are not shared due to privacy restrictions.

SUPPORTING INFORMATION

The following additional material may be found online:

Appendix S1: 15-item criteria checklist.

Appendix S2: Topic list of the larger study.

Figure S1: Overview of inclusion of participants.

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PERSPECTIVAS DE LOS PADRES SOBRE EL TRATAMIENTO CON NUSINERSEN PARA NIÑOS CON ATROFIA MUSCULAR ESPINAL

OBJETIVO

Obtener una idea de las perspectivas de los padres sobre su proceso de toma de decisiones sobre el tratamiento nusinersen para su hijo, incluidas las necesidades y preocupaciones percibidas, y explorar los factores que influyen en este proceso.

MÉTODO

Este fue un estudio exploratorio de entrevistas cualitativas entre padres de niños con atrofia muscular espinal tipos 1 a 3. Los datos se analizaron mediante análisis temático inductivo.

RESULTADOS

Participaron diecinueve padres de 16 niños que representan a 13 familias. Se informó una amplia variedad de perspectivas que van desde un enfoque biomédico, que se centró en la lucha contra la enfermedad, hasta un enfoque holístico, que apunta a una buena calidad de vida para su hijo. Los factores más importantes que ayudaron a los padres a decidir fueron la comunicación honesta y neutral con su médico y el acceso a la información disponible.

INTERPRETACIÓN

Es importante que los médicos comprendan que existen diferentes perspectivas que influyen en el proceso de toma de decisiones. Los médicos deben crear un entorno que les permita a los padres aceptar o rechazar el tratamiento comunicándose honesta y abiertamente con ellos y discutiendo ampliamente ambas opciones. Se debe proporcionar información clara sobre los pros y los contras, los avances recientes en la investigación y las experiencias de otros padres para que los padres puedan tomar una decisión informada.

PERSPECTIVAS DOS PAIS SOBRE O TRATAMENTO COM NUSINERSEN PARA CRIANÇAS COM ATROFIA MUSCULAR ESPINHAL

OBJETIVO

Obter informações sobe as perspectivas dos pais sobre seu processo de tomada de decisão relativa ao tratamento com nusinersen para sua criança, incluindo necessidades e preocupações percebidas, e explorar fatores que influenciam este processo.

MÉTODO

Este foi um estudo exploratório com entrevista qualitativa entre pais de crianças com atrofia muscular espinhal tipos 1 a 3. Os dados foram analisados usando análise temática indutiva.

RESULTADOS

Dezenove pais de 16 crianças representando 13 famílias participaram. Uma ampla variedade de perspectivas foi reportada, variando de uma abordagem biomédica, que focou no combate à doença, a uma abordagem holística, que visava uma boa qualidade de vida para a criança. Os fatores mais importantes que ajudaram os pais a decidir foram a comunicação honesta com o médico e acesso a informação disponível.

INTERPRETAÇÃO

É importante que médicos compreendam que há diferentes perspectivas influenciando o processo de tomada de decisões. Os médicos devem criar um ambiente que permita aos pais aceitar ou rejeitar o tratamento por meio de comunicação honesta e aberta com eles, e por meio de discussão extensiva de ambas as opções. A informação clara sobre os pós e contra, desenvolvimentos recentes de pesquisas, e experiências de outros pais deve ser disponibilizada para permitir aos pais tomar uma decisão informada.