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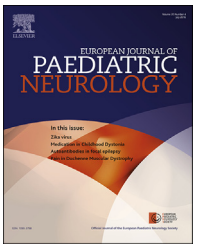

Original article

Information and treatment decisions in severe spinal muscular atrophy: A parental follow-up

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ABSTRACT

Introduction: The parents of children with severe spinal muscular atrophy (SMA) face difficult ethical decisions regarding their child's treatment. This study explored the experience of parents of children with severe SMA concerning information and treatment decisions.

Material and methods: This nationwide survey, conducted in 2013, is based on parents of children who were born in Sweden between 2000 and 2010 and later diagnosed with SMA type I or II where respiratory support was considered the first year of life (N = 61, participation rate: 87%). The survey involved parents' perception of the child's care and the questions used in this study covered information given and treatment decisions. Descriptive statistics were used.

Results: None of the parents reported that the health care professionals made decisions concerning the child's treatment without informing them first, and 80% reported feeling confident about the decisions made. Of the bereaved parents, 11/48 (23%) reported that they got no information about respiratory support, compared to 2/13 (15%) of non-bereaved. Bereaved parents were more likely to report being satisfied with and understanding the information given about the illness and its treatment than non-bereaved parents.

Conclusion: All parents reported having been informed before treatment decisions were made and a vast majority reported feeling confident about the decisions. However, a quarter of the parents declined to have received information about respiratory support, which indicates that the parents did not sufficiently understand the available respiratory treatment options, and that their children may not receive the kind of care that is recommended in guidelines.

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1. Introduction

Spinal muscular atrophy (SMA) is the second most common autosomal recessive disease, with an incidence of 1 in every 6000–10,000 live births.[1] SMA affects motor neurons and exhibits various progression of muscle weakness. Based on age at presentation and severity, SMA is classified into four grades of severity. SMA type I is the most severe form and by definition presents during the first 6 months of life. Death usually occurs within the first 2 years of life without respiratory support, making SMA the second most common lethal autosomal recessive disease. SMA type II presents slightly later than type I, at 6–18 months, and is associated with a risk for shortened life expectancy. SMA type III causes symptoms after 18 months of age, and the life expectancy is generally in line with that of the general population. SMA type IV, finally, presents in the second or third decade of life, and walking ability is retained during adult years.[1,2]

Despite promising novel therapeutic drugs in the pipeline for SMA, including the most severe forms, there is presently no cure for any form of SMA. Supportive and palliative care are therefore cornerstones in the treatment of severe SMA, focusing on preventing complications of weakness and maintaining quality of life. Key issues here involve respiratory and nutritional support.[3] Life can be considerably prolonged for children with severe SMA by introducing respiratory support. However, the impact of such support on the child's wellbeing is unknown, and there is no evidence or consensus among experts on the use of respiratory support for SMA type I. In the absence of consensus, Wang and co-workers[4] developed care guidelines for SMA based on evidence and a Delphi consensus-building process. Their guidelines concluded that caregivers should explore treatment options together with the family in relation to the child's potential, quality-of-life issues, and family desires. This need is particularly evident and important regarding the use of respiratory support.[4] A key issue is to what extent respiratory support affects the quality of life of the child with severe SMA, and recommendations vary between clinics and clinicians. Some favor the use of respiratory support for SMA type I,[5–7] while others strongly oppose its use,[8,9] arguing that the burden for the child and family is greater than the benefit. The international care guidelines from 2007 consider such differences in treatment recommendations, and conclude that clinicians have an obligation to present treatment options to the parents in an open, fair and balanced manner.[4]

Although there is evidence from other severe childhood diseases that parent information and participation in decision making has a high impact on the end-of-life care and parents' and children's wellbeing,[10–12] little is known about the situation for parents of children with severe SMA. The few existing studies on SMA[13–15] have examined parents' experience of having a child with SMA but from a different perspective. Higgs and co-workers[15] interviewed 13 bereaved parents from seven families about their experiences of having a child with SMA type I. These parents described the shock, the anticipatory grief, the helplessness, but also that they were grateful for being given control over decisions, e.g. about how and where their child died. In the interview study by Parker, Maddocks and Stern,[14] nine bereaved and four non-bereaved family members of individuals with muscular dystrophy or SMA perceived a lack of coordination of care and competent health care professionals, inadequate bereavement care, and limited discussions of options for respiratory support and advance directives.

When a child is diagnosed with severe SMA, parents and caregivers are confronted with many difficult treatment decisions and emotionally demanding situations. In order to arrange care in accordance with the families' wishes and concerns, we need more knowledge about the parents' perspective on the child's care. Therefore, we have carried out a nationwide study to explore the experience of non-bereaved and bereaved parents of children with severe SMA, where respiratory support was considered during the first year of the child's life, concerning information and treatment decisions.

2. Material and methods

This study is based on a nationwide survey, conducted in 2013, of the parents of children who were born in Sweden between the years 2000 and 2010 and later diagnosed with SMA type I or II, and for whom respiratory support was considered by the health care professionals during the first year of life. The children (N = 40) were identified through the National Board of Health and Welfare and their parents/guardians through the Swedish Tax Agency (N = 78) (Fig. 1). Altogether, 70 parents of 37 children were contacted by phone and asked about their willingness to participate. Fourteen parents of seven children who were still living with SMA (here called non-bereaved parents) and 56 bereaved parents of 30 children were sent a letter of invitation in February 2013. In all, 61 parents of 36 children participated (participation rate: 87%). Parents of all eligible children except one are represented in the study.

The questionnaire was mailed to the parents who agreed to participate. To maintain anonymity, the parents returned the questionnaire separately from a reply card where they stated their name. This made it possible to keep track of those who returned the questionnaire and those who needed to be reminded. Ten days after the questionnaires had been sent out, a combined thank-you and reminder card was mailed to parents who had agreed to participate. If the parents did not return the response card, they were phoned to ask if they had any particular concerns or needed assistance filling out the questionnaire.

The questionnaires, one for non-bereaved parents and one for bereaved parents, were developed using a stepwise approach described by Charlton[16] and others.[17–19] First, non-bereaved and bereaved parents of children with SMA were interviewed. They were asked to describe their overall experience of their child's care and more specifically about information and treatment decisions. Second, themes in the parents' narratives were identified and questions were elaborated accordingly. Third, questionnaires were developed and refined with input from health care professionals. Face-validity testing of the questionnaires was performed with

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