Brief Communication

Respiratory support attitudes among pediatric intensive care staff for spinal muscular atrophy patients in Saudi Arabia

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ABSTRACT

Objectives: To explore therapeutic attitude of healthcare providers practicing in pediatric critical care in Saudi Arabia toward patients with Spinal Muscular Atroph (SMA) Type I, and to explore their awareness about the International Consensus statement for SMA care.

Methods: A cross-sectional survey was conducted in April 2015 during 6th Saudi Critical Care Conference, targeting physicians and respiratory therapists practicing in Pediatric Critical Care.

Results: Sixty participants accepted to participate in this survey. Out of those who answered the questionnaire, 44 were included in the analysis. Majority (66%) of participants were unaware of the International Consensus guidelines for SMA. Endotracheal intubation was reported as an acceptable intervention in SMA patients with acute respiratory failure by 43% of participants. Similarly, chronic home ventilation was agreed by 41% of participants.

Conclusion: A nationwide adaptation of the International SMA Consensus guidelines for children with SMA I is recommended, aiming to decrease variability and standardize their management across various healthcare facilities in Saudi Arabia.

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Spinal Muscular Atrophy (SMA) is a congenital, autosomal recessive disease characterized by mutations in the survival motor neurons gene on chromosome 5, which leads to degeneration of the anterior horn cells of the spinal cord and the motor cells of the cranial nerve nuclei.[1] The SMA Type I is the most severe form, with an estimated incidence of 1 in 6,000 to 10, 000 live births.[2,3] It is the leading genetic cause of death in infancy, with a reported life expectancy of fewer than 2 years.[4] The onset of symptoms occur within the first few months of life; with increasing muscle weakness, hypotonia and bulbar involvement leading to lung and chest wall under development, hypoventilation, impaired cough and inability to clear airway secretions. Without ventilator support, these patients die ultimately from respiratory insufficiency, the primary cause of mortality in these patients.[5]

A large spectrum of options can be offered for these patients regarding the respiratory support, ranging from either no ventilatory support or emphasis on palliative care, or providing more respiratory support, either by non-invasive ventilation or invasive mechanical ventilation, with or without tracheostomy.[6,7] Multiples healthcare centers in Saudi Arabia offer variable interventions to patients with neuromuscular diseases, and these interventions may prolong the survival into the second decade of life for SMA I patients. For the treating teams, difficult issues may arise concerning the appropriate goals of treatment and the ethics of offering or withholding life support therapies for SMA I patients.[6]

The purpose of this study is to explore the therapeutic attitude of healthcare providers working in the pediatric critical care domain in Saudi Arabia toward patients with SMA Type I. Another aim was to explore their awareness of the recommendations of the International Consensus statement for standard of care in SMA.[8]

Methods. This is a cross-sectional questionnaire-based survey that was conducted in April 2015. The inclusion criteria were physicians and respiratory therapists working in the domain of Pediatric Critical Care in Kingdom of Saudi Arabia. Other healthcare providers working in other critical care setups were excluded. The survey was distributed during the 6th Saudi Critical Care Conference. Attendees to Pediatric track of this scientific gathering were invited to fill the survey. To improve the recruitment process and to reach for all the target population who may have not been in the conference venue, we circulated an email via Saudi pediatric critical care e-mailing group, followed by 2 reminders within 4 weeks.

The questionnaire was drafted by the authors based on a review of the literature regarding SMA Type I ventilatory support and end of life care. Then a

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multidisciplinary team focus group meeting was utilized to produce the final version. Experts from our pediatric critical care unit reviewed the questionnaire. It was then piloted in our pediatric intensive care unit healthcare workers, and tested to ensure and determine its clarity, before sending it to the targeted group. The study intended to investigate the participant's therapeutic attitude toward respiratory support and acceptable interventions for acute and chronic respiratory failure in SMA I patients, to explore their awareness of the recommendations of the Consensus statement for standard of care in SMA.

The questionnaire was divided into 3 parts. The first part included the demographic variables of the respondents including years of experience, specialty, credential, gender, employment sector (whether university, governmental or private hospital setting) and number of beds in their hospital. The second part enquired on their awareness of the guideline of the International consensus statement for standard of care in SMA. The third part explored their therapeutic attitude and their position toward respiratory support and acceptable interventions for acute and chronic respiratory failure, namely: whether they agree, disagree or if they have a neutral position for each of the following statements: If endotracheal intubation is acceptable in acute exacerbations for SMA type I patients, whether chronic home ventilation is acceptable for SMA type I patients, if treating team should offer tracheostomy for SMA type I patients, and whether to consider withholding mechanical ventilation from SMA type I patients.

This study received prior ethical approval by the Institutional Review Board (IRB) of King Saud University (IRB # E15-1476). Statistical analysis for the data was carried out and the categorical variables were expressed as percentages.

Results. Out of 82 staff who were approached, 60 (73%) of them accepted to participate in the survey and answered the questionnaire, but 16 of them were excluded because of incomplete answer of the survey questions. The demographic characteristics of the participants are shown in **Table 1**.

Physicians' attitude toward respiratory support and acceptable interventions for acute and chronic respiratory failure for SMA Type I are summarized in **Table 2**. In this sample, 66% of the participants were not aware of the International Consensus guidelines for standards of care for SMA. When answering if endotracheal intubation is an acceptable intervention for SMA Type I patients who are in acute respiratory failure, 43% of the participants agreed, 36% disagreed to proceed with this intervention and the remaining had a neutral position. Participants who agreed for chronic home ventilation (41%) were marginally more than those who disagreed (36%), while the rest (23%) had a neutral position.

Regarding tracheostomy in SMA I chronically ventilated patients, whether to offer this option for the family or not, 48% of participants stated their refusal to offer this option while 38% agreed for it. On the other hand, the participants who refused the decision to withhold the mechanical ventilation for these patients were slightly higher (54%) than those who supported this decision (46%).

Discussion. This study characterized the attitude of the health care providers working in the pediatric critical care domain in Kingdom of Saudi Arabia towards SMA type I patients. This study revealed that only 30% of the respondents were aware of the consensus guidelines for management of SMA type I patients. Moreover, significant variation was noticed regarding their therapeutic attitudes in both situations for acute respiratory exacerbation and for chronic respiratory support.

These variations in the management decisions among participants could be explained by the fact that, treatment options and modalities of SMA type I patients are quite challenging due to paucity of internationally recognized practice guidelines for those patients. Another important factor for such variability could be attributed to the variation in knowledge resources and the difference in centers at which participants were trained and consequently they inherited its common

Table 1 - Demographic characteristics of the participants. n=44

Variables	n (%)
Gender	
Male	32 (72.7)
Female	12 (27.3)
Credential	
Consultant	20 (45.5)
Other physicians*	15 (34)
Respiratory therapist	9 (20)
Hospital setting	
Tertiary	37 (84)
Secondary	7 (16)
Type of hospital	
Governmental	40 (90)
Private	4 (10)

Includes fellows, specialists & residents

Table 2 - Comparison of statements from "Consensus Statement for Standard of Care in Spinal Muscular Atrophy"[8] to the surveyed participants' responses toward different respiratory support statements for SMA type I.

Consensus statement Recommendation	Statement asked in the survey	Consultants			Others		
		Disagree	Neutral	Agreed	Disagree	Neutral	Agreed
		n (%)					
"If noninvasive ventilation fails, nonsitters may be intubated and mechanically ventilated as a short-term measure"	"In acute exacerbation, endotracheal intubation is acceptable for SMA type I patients"	9 (45)	4 (20)	7 (35)	7 (29)	5 (21)	12 (50)
"If noninvasive ventilation was needed during an acute illness, home noninvasive ventilatory support should be considered."	"Chronic home Ventilation is acceptable for SMA type I patients"	8 (40)	4 (20)	8 (40)	8 (33)	6 (25)	10(42)
"In nonsitters with frequent respiratory infections, tracheotomy and ventilation can be considered but may not improve quality of life or decrease hospitalizations"	"Treating team should offer tracheostomy for SMA type I patients"	12 (60)	3(15)	5 (25)	9 (37)	3 (13)	12 (50)
"In nonsitters, care without ventilation support is an option if the burden of management outweighs benefit. Noninvasive ventilation may be used palliatively to facilitate hospital discharge and reduce work of breathing."	"Will you consider withholding Mechanical Ventilation from SMA type I patients?"	8 (40)	--------	12 (60)	16 (67)	--------	8 (33)

practice in managing such patients. These factors might result in a broad range of therapeutic options regarding treatment of respiratory failure among such patients and consequently, the same child's treatment plan may vary from simple palliative care to extensive respiratory support from one care giver to another.[6,9]

The major accounted milestone was in 2005; when the international standards of care committee for SMA patients was established, and the first International Consensus guidelines were published in 2007.[8] The aim of this Consensus guidelines was to reduce the variability of care for SMA type I patients and standardize the therapeutic attitude of health care providers caring for such patients.

These findings are similar to Benson et al[10] who conducted an international survey regarding the evntilatpry support attitude towards SMA type I patients, and they reported that only 50% of the respondents were aware of the International Guideline Consensus. Moreover, their survey confirmed the existence of regional differences with regard to the physician's recommendations for chronic invasive ventilation and wide variability existed even within the same region.[10]

In this study, the greatest practice variability was noticed regarding the decision whether to offer (or not) the tracheostomy option for the family. Some variability was also noticed among the respondents regarding the decision to withhold the mechanical ventilatory support, as more respondents did not agree on this approach. This diversity of therapeutic attitudes may be partially due to the lack of awareness of the the Consensus Statement for Standard of Care in Spinal Muscular Atrophy guidelines, in addition to other potential influencing factors, such as cultural differences, religious concerns, and variable administrative and legal issues. Moreover, absence of a known curative therapy for such patients, at the time of survey, might influence the decision of some participants regarding their attitude for the invasive intervaentions in the management of those patients.

Consequently, those patients are being offered different therapeutic options and recommendations in different regions of Kingdom of Saudi Arabia, and this could be another challenge for both the families and the physicians regarding the extent of care for their patients.

This study has been conducted in Kingdom of Saudi Arabia, with its potential benefit to emphasize the major need to standardize the management of SMA I patients across the country. Moreover, more education and updates in the International SMA Consensus guidelines should be conducted and distributed to the physicians working in the domain of Pediatric Critical Care as they are frequently involved in the management of SMA I patients where they can be the primary decision maker for them in different situations. Therefore, their opinion and input regarding these patients may significantly influence the clinical practice and the family decision.

Our study has several limitations, such as the low response rate, however, busy intensivists in these acute critical care settings need to share even such limited responses, as this may highlight the situation further for them, especially for SMA patients who present

with further respiratory compromise, and families may request further opinion from other local practices and other management options. Our study also has potential selection bias; as the targeted participants were from healthcare providers attending the 6th Saudi Critical Care Conference, however, this was minimized by circulating the survey via Saudi pediatric critical care e-mailing group. Another limitation is that the responses might not correlate with the real practice of the respondents in some situations, as we assessed only their opinion. Patient's management is usually individualized on case-by-case assessment, so differences may arise between the opinion and the actual practice.

Future directions. This may be addressed by establishing a national registry for SMA cases. Besides that, conducting a similar questionnaire among pediatricians and neurologists in Saudi Arabia, with more specific details on chronic management and further exploration of the social, religious and resources components, will further highlight the chronic care, considering the ongoing clinical trial for new investigational therapies for SMA.[11]

In conclusion, this study highlights the limited awareness of health care providers managing SMA type I patients in Kingdom of Saudi Arabia for the International SMA Consensus guidelines for children with SMA I. Therefore a nationwide adaptation of these guidelines is recommended, aiming to decrease variability and standardize the management across various healthcare facilities in Saudi Arabia. More education should be considered to conduct and distribute the guideline to the healthcare providers working in the domain of Pediatric Critical Care across the country.

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