

17. Jn 15:5, "*1 am the vine; you are the branches. If a man remains in me and I in him, he will bear much fruit; apart from me you can do nothing.*"

Management of Chronic Neuromuscular Diseases in Children

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Physicians caring for children with severe neuromuscular disorders are often faced with decisions concerning how aggressive the therapy should be. Before the widespread use of mechanical ventilators, no effective treatment was available for weakness developing in breathing muscles, and the children subsequently died because of respiratory failure. With the introduction of mechanical ventilators and other forms of advanced technology, physicians were placed in situations where they had to deal with complex medical and ethical issues. Traditionally, the decisions of how to manage these difficult problems were based on discussions between the physician and patient and/or parent. However, each new advance in technology has extended the ethical scope of the decisions.

In this paper we deal with three related questions.

1. What is the prevailing practice in regard to management of these difficult problems?

2. What treatment is medically or technically feasible?

3. What is the Biblical mandate for management of these patients?

Patient-care issues other than mechanical ventilation are quite pertinent to these children, but our discussion will focus on the use of this modality.

METHODS

A questionnaire assessing the level of care physicians would choose to give patients with various forms of neuromuscular disease was sent by mail to 25 child neurologists (CN) and 26 pediatric critical care specialists (CCS). The subjects were senior physicians who were recognized experts in their specialties and were not chosen by random. Completed questionnaires were returned by 21(84%) CN and 15 (58%) CCS.

The questionnaire consisted of seven

vignettes with forced choice responses assessing the level of care the physician would deliver to each patient. The responses were measured in ordinal scales ranging from either I to 3 or I to 4. In cases where the subject checked more than one response item to a vignette, the mean score of the two items was used as their response (e.g. 2 and 3 = 2.5).

The differences between the CN and CCS in their responses to each vignette were tested with Kruskal-Wallis Analysis of Variance Tests for Ranked Data.' The data in the Table are presented as mean ranks so that the higher the mean rank score the more aggressive the choice of therapy.

RESULTS

In the first three vignettes dealing with infants and young children, there were no significant differences in the therapy offered by CN or CCS. The physicians appeared to increase the level of care as the patient increased in age. For example, in the first vignette dealing with a newborn suspected of having spinal muscular atrophy who developed respiratory failure (Table), 14.3% of the CN and 26.7% of the CCS recommended the use of mechanical ventilation ($p > 0.05$). In the second vignette describing a bright two-year-old with spinal muscular atrophy whose breathing becomes compromised without evidence of pneumonia, 40% of the CN and 50% of the CCS physicians would choose mechanical ventilation ($p > 0.05$). In contrast, in a bright two-year-old with spinal muscular atrophy who develops pneumonia and respiratory failure, 33.3%

of the CN and 66.7% ($p > 0.05$) of the CCS would add mechanical ventilation, if necessary, to the standard pneumonia treatment of IV fluids and antibiotics.

For the two vignettes (5 and 6) describing adolescents who develop respiratory failure following pneumonia, the CCS physicians recommended significantly more aggressive treatment than the CN's. For a bright 19-year-old female with spinal muscular atrophy, 73.4% of the CCS compared to 38.1% of the CN ($p < 0.032$) would continue mechanical ventilation indefinitely, if necessary. For a dull normal 16 year-old boy with Duchenne muscular dystrophy 46.6% of the CCS and 9.5% of the CN ($p < 0.009$) recommended continuing mechanical ventilation as long as necessary. In contrast for a nine-year-old child (number 7) with spinal muscular atrophy and an IQ of 35 who develops pneumonia and respiratory failure, 70% of the CN and 46.7% of the CCS would limit treatment to antibiotics and fluids.

The fourth vignette described a two-year-old child with pharyngeal muscle weakness who has had several episodes of aspiration pneumonia due to his severe swallowing difficulties. For this child 94.3% of the CCS physicians would recommend the most aggressive therapy option offered of surgical insertion of a feeding device, as compared to 60.9% ($p \leq .034$) of the CN's.

Fourteen CN and 13 CCS wrote explanatory notes in addition to (or in 3 cases, rather than) answering the

questionnaire. Two themes ran through their comments. Fourteen physicians emphasized the importance of discussing alternatives with the family and/or patient. Seven (six CN and one CCS) outlined the general principle of avoiding mechanical ventilation in this group of patients.

The questionnaire itself and detailed results are available on request from the authors.

DISCUSSION

The most apparent result from the questionnaire is that many specialists dealing with children having neuromuscular disorders would opt against mechanical ventilation in circumstances where death is the likely outcome without mechanical support. CCS tended to choose long-term ventilation with greater frequency than CN. Many responders additionally noted the importance of explaining the alternatives to the patient and family.

Two other factors seemed to influence the choice of care. As the age of the patient increased, more vigorous therapy tended to be applied. This may relate to the technical difficulties with mechanical ventilation in very young patients or to the fact that older children have demonstrated their potential to survive. Additionally, a lower cognitive level of the patient also appeared to result in mechanical ventilation being offered less frequently. The structure of the questionnaire, however, did not allow this point to be examined statistically.

The long-term use of mechanical ventilation clearly is a medical alternative in older

patients with Duchenne muscular dystrophy and other less common dystrophies, but it is not the most commonly chosen one. Colbert and Schock sent questionnaires to directors of Muscular Dystrophy Association clinics.² Approximately one-third of the clinics routinely prescribed respiratory support with a wide variety of negative and positive pressure ventilators for these patients. They stated, "some physicians hesitate to prescribe ventilatory aids because they see prolongation of life for people with progressive disease as increasing suffering and placing an unnecessary burden on the families or caretakers. Other doctors readily offer such support systems with the assumption that their patients can continue to enjoy a meaningful life". Alexander et al. employed long-term mechanical assistance in ten patients with Duchenne muscular dystrophy.³ Mean survival after adding assistance was 3.4 years. Bach et al. reported 29 muscular dystrophy patients who required mechanical ventilation 24 hours per day.⁴ Their average age was 27 years with a range of 15-54 years. Twenty-four of those individuals lived in the community. Three were married with a total of five children. Several were involved in professional careers. Interviewing five young men who were totally dependent on respirators, Gilgoff wrote, "When asked if they wanted a no resuscitation order to be added to their charts should they arrest, all were adamantly opposed".⁵

Less experience is available in young children or infants with spinal muscular atrophy. Although older children with

spinal muscular atrophy surviving to later ages without mechanical ventilation can be approached in the same manner as older children with muscular dystrophy, very young children with spinal muscular atrophy present unique problems. First, they are usually too young to participate in any decisions. Secondly, there is much less experience in maintaining these individuals with mechanical ventilation. Splaingard et al. listed two patients, 6 months and 1-1/2 years, with spinal muscular atrophy, who received mechanical ventilation and survived for extended periods.⁶ However, details of their courses were not provided. In general, far less experience is available in this area, and the technical problems, such as maintenance of tracheostomy in a small, growing patient, may be nearly insurmountable.

From this comparison of prevailing views among medical specialists dealing with this area and what is technically feasible, a picture of a wide gap emerges. More extensive treatment is available and possible than is recommended by the majority of physicians. The responding physicians are well-acquainted with the full character of these difficult problems, and the number of technical options available may exceed the ethical ones. Nevertheless, in consideration of the great variability of views among the physicians themselves, it is all the more important that parents be knowledgeable about the medical options as they really exist. In these decisions, there is no reason to conclude that the physician's opinion is automatically better-founded than the parent's. Although our questionnaire was designed to assess

physicians' views and not those of parents, many specialists added comments indicating the importance of an informed parent.

Issues concerning the older child with neuromuscular disease operate in the context of a patient who participates in decisions. Mechanical ventilation in patients with these disorders may not be merely prolongation of the act of dying; life may be prolonged for months or years. The physician and family must know a great deal about the course of the illness with respirator therapy. Is the process of dying only to be extended by painful, undignified means, or is the possibility of prolonged life to be held out? There is no Biblical mandate for prolonging suffering, but the extension of life as created by God is to be honored. In order to withhold extraordinary treatment, Koop said, "I must know an extraordinary amount about the disease process under consideration. I must know an extraordinary amount about my patient. I must know an extraordinary amount about the reaction of my patient to the disease process in question."⁷

The decision to withhold treatment or extend life is not a theoretical one. The first brings about an irrevocable conclusion, and the second requires an on-going commitment under often difficult circumstances. The overriding Scriptural command should be: "Thou shalt love thy neighbor as thyself (Mark 12:31).⁸ How best to follow this command may not always be clear, but the possibility of extended prolongation of life seems to offer the greatest potential for

demonstrating this love. Cordon Clark emphasized the intellectual and volitional aspects of such matters of the heart; clearly the response to a command is not based in the emotions.⁹ Fulfilling the command in this circumstance involves more than a decision to persist with therapy. Continuing action is required. This may mean not only involvement with the particular patient but also the establishment of large scale, costly programs to support management of these patients.

Is the older patient free to choose against continuing life? The courts usually will respect the wishes of a patient not to accept a particular form of therapy. However, Scriptural commands are always offered in the context of a living individual, and it is difficult to see how the patient can be obedient to these commands by choosing death. The presence of a severe illness does not absolve the patient of the responsibility of obedience. Job wanted death because of his illness (Job 10:18), but was reminded that God was in control. The concept of a "meaningful" life pales in comparison to God's creation and sovereignty; the idea that a patient's life must have "meaning" in order to be preserved becomes muddy in this context. The general principle of choosing life over death is most important, but it must be admitted that it is impossible to include all circumstances under this rubric. These exceptions, however, do not affect the rule.

With the younger child the medical details are even less clear, and the parents must take the responsibility for decisions (Col

3:20). This seems to be the general, public view. The Presidents' Commission stated, "Public policy should resist state intrusion into family decision-making unless serious issues are at stake and the intrusion is likely to achieve better outcomes without undue liabilities."¹⁰

Addressing the issue of treatment for seriously ill newborns, the Commission noted that the presence of "permanent handicaps justify a decision not to provide life-sustaining treatment only when they are so severe that continued existence would not be a net benefit to the infant."¹⁰ The Commission observed that this strict standard excludes consideration of the possible adverse affects of the child's life on the rest of the family, society, etc. This is an exceedingly important principle and would seem to apply to young patients with neuromuscular diseases. The basis of this principle is the sixth commandment (Exodus 20:13). Considered in this context, the rationale for withholding therapy because of the patient's cognitive impairment is very weak. To summarize, the Biblical commandments for love, the extension of mercy, and the prohibitions of the sixth commandment against murder are essential considerations for young children who would die without mechanical ventilation. These commands should override consideration of the technology available and the effects of the decision external to the patient. Parents may benefit greatly from seeking the counsel and prayers of wise elders (Jas 5:14).

Other considerations should include the prohibition against lying (Exodus 20:16) to

the patient, appropriate application of anointing with oil and prayer for healing (Jas 5:13-16), and the state of the patient in respect to salvation. Any decision should provide latitude for the supernatural work of the Holy Spirit both for physical and spiritual healing. For example, it would seem that great efforts should be made to maintain life in an older child who is not regenerated. "Life" is mentioned many places in the Scriptures, but a large percentage of these references pertain to eternal or everlasting life with God and not

to life on the present earth.

Finally, there is no logical or Biblical distinction between not starting and discontinuing a clearly non-beneficial treatment. To make this distinction amounts to domination by the technology of the situation. Although we may have emotional restraint about stopping a particular therapy that has been started, the guiding principle is still the Biblical command to love the patient.

TABLE:

	Child Neurologists	Pediatric Critical Care Specialists	
Mean Ranks of Level of Aggressiveness in Therapy Chosen by Child Neurologists and Pediatric Critical Care Patients	MEAN RANK	MEAN RANK	P
1. An extremely hypotonic newborn is delivered to a family where a previous child has died of spinal muscular atrophy. Muscle biopsy at one week of age, along with electromyography, is consistent with spinal muscular atrophy. The infant is alert and there has never been any indication of hypoxia. In the second week of life, the infant begins to develop indications of respiratory failure. It becomes clear that intervention would be required to maintain life.	18.24	18.87	NS
2. You are following a two-year-old with spinal muscular atrophy in your practice. Although acquisition of motor skills has been minimal, the child is quite bright and speaks in sentences. Over the course of a few weeks, the breathing becomes compromised. Chest x-ray does not reveal any evidence of pneumonia.	16.92	18.32	NS
3. A bright, two-year-old child with spinal muscular atrophy develops pneumonia and respiratory failure.	16.60	21.17	NS
4. A two-year-old child with congenital myopathy has severe swallowing difficulties and has developed several episodes of aspiration pneumonia secondary to pharyngeal muscle weakness.	15.00	20.67	0.034
5. A nineteen-year-old girl has had muscle weakness since early infancy with a diagnosis of spinal muscular atrophy. The child has never walked. However, the school performance has been excellent. She develops pneumonia and respiratory failure.	15.55	22.63	0.032

6. You are following a sixteen-year-old boy in your practice with Duchenne muscular dystrophy. His intelligence is dull normal. He develops pneumonia and respiratory failure.	14.81	23.67	0.009
7. A nine-year-old child with spinal muscular atrophy develops pneumonia and respiratory failure. The child has previously had an episode of respiratory failure with hypoxia. That episode left the child with severe cognitive deficit (IQ 35).	16.27	20.30	NS

References

- Hull, CH, Nie, NH: *SPSS Update 7-9*. McGraw Hill Book Co., New York, pp. 237-239, 1981.
- Colbert AP, Schock NC: Respirator use in progressive neuromuscular diseases. *Arch Phys Med Rehabil* 66: 760-762, 1985.
- Alexander MA, Johnson EW, Petty J, Stauch D: Mechanical ventilation of patients with late stage Duchenne muscular dystrophy: management in the home. *Arch Phy Med Rehabil* 60: 289-292, 1979.
- Bach J, Augusta A, Pilkington LA, Lee M: Long-term rehabilitation in advanced stage of childhood onset, rapidly progressive muscular dystrophy. *Arch Phys Rehabil* 62: 328-331, 1981.
- Gilgoff IS: End stage Duchenne patients: choosing between respirator and natural death. Chap 34 in *Psychosocial Aspects of Muscular Dystrophy and Allied Diseases*, ed. LI Charash, SG Wolf, AH Kutscher, RE Lovelace, MS Hale. Charles C. Thomas, Publisher, Springfield Illinois, 1983.
- Splaingard ML, Frates RC, Harrison GM, Carter RE, Jefferson LS: Home-positive pressure ventilation. Twenth years experience. *Chest* 84: 376-382, 1983.
- Koop CE: *The Right to Live, The Right to Die*. Tyndale House Publishers, Inc., Wheaton, Illinois, 1980.
- Payne, FE: *Biblical/Medical Ethics - The Christian and the Practice of Medicine*. Mott Media, Milford, Michigan. 1985. p. 68.
- Clark, GH: *The Biblical Doctrine of Man*. Trinity Foundation, Jefferson, Maryland. 1984. pp. 78-88.
- President's Commission for the Study of Ethical Problems in Medicine and Biomedical and Behavioral Research. Deciding to forego life-sustaining treatment. March 1983. U.S. Government Printing Office, Washington, DC. p. 218.