

Spinal muscular atrophy type 1 quality of life.

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OBJECTIVE: To compare healthcare professionals' assessment of the quality of life of spinal muscular atrophy type 1 children with that of the care providers for the children. **DESIGN:** The care providers of all 53 surviving spinal muscular atrophy type 1 children managed in one neuromuscular disease clinic were sent Likert-scale surveys of six quality of life issues and ten polar-adjective pairs. The quality of life estimations were compared with those of 67 clinicians and with those of 30 parents considering their unaffected children. **RESULTS:** One hundred care providers from 46 out of the 53 families (87%) responded. Although the clinicians' mean estimate of the children's quality of life was 2.85 +/- 0.2/10, the care providers' estimate was 7.81 +/- 0.2/10 ($P < 0.0001$). The care providers also found life with the children to be satisfying (6.0 +/- 0.2/7), interesting (6.6 +/- 0.1/7), friendly (6.1 +/- 0.1/7), enjoyable (6.3 +/- 0.1/7), worthwhile (6.7 +/- 0.1/7), full (6.6 +/- 0.1/7), hopeful (5.9 +/- 0.2/7), and rewarding (6.4 +/- 0.1/7), and they estimated the children to be happy (8.5 +/- 0.2/10) and their lives worth living (9.6 +/- 0.1/10). However, 69 of 104 felt that their lives were hard rather than easy, and 56 of 104 reported feeling tied down rather than free. Although the effort they felt for raising the child was high (8.3 +/- 0.3 by comparison with 5 for an unaffected child), the burden they felt in doing so was not (5.8 +/- 0.3/5). When asked whether they would or would not recommend ventilator use, 31 clinicians (45.5%) indicated they would, 24 (36.4%) would not, and 12 (18.2%) chose not to respond to this question. Care provider responses did not differ significantly from the responses of the parents of unaffected children except for the easy/hard semantic differential (care providers, 3.80 +/- 1.75 controls, 5.27 +/- 1.14, < 0.001). **CONCLUSIONS:** Although there is a widespread perception that spinal muscular atrophy type 1 children have a poor quality of life, this perception is not shared by their care providers.