A Difficult Case

Home ventilation of a child with motor and sensory neuropathy

R H Davies

A paediatrician describes a young child who was eventually diagnosed as having autosomal recessive hereditary motor and sensory neuropathy type III and the uncertainties surrounding the decision to support the child at home on domiciliary ventilation. The child's mother gives her reaction to the decision, and we also invited comments from a public health physician, a nurse, and an intensive care paediatrician.

Case report

The child, a girl, was born at 37 weeks' gestation by elective caesarean section because of severe intrauterine growth retardation. Her birth weight was 1830 g. She spent the first two weeks in a special care baby unit, and there was difficulty in establishing feeding. She smiled at 9 weeks and was aware of her environment and people. Throughout her life her cognitive development was always normal. However, from 3-6 months of age she made no attempt to lift her head or to roll and she could not sit even when supported. She appeared to feed normally, but at 6 months she was failing to thrive. She was the third of her parents' three children. There was no family history of neurological disease.

The child was not known to be seriously unwell until she was admitted to a district general hospital paediatric ward when 6 months old with respiratory distress. Pneumonia was diagnosed and confirmed by chest radiograph. Then it became apparent that she had widespread muscular weakness and that she was in respiratory failure because of weakness of the respiratory muscles. She was admitted to the intensive care unit, paralysed, sedated, and ventilated. An underlying neuromuscular problem was diagnosed, and she was transferred on a ventilator to the regional university hospital for evaluation two days after her admission.

"The home treatment of this child ...was a costly but beautiful deed"

There she was found to have weakness of most, but not all, movements. At first tendon jerks were present, but they and the muscular movements steadily diminished. The clinical picture of a severe and progressive peripheral neuropathy was confirmed by nerve conduction studies, but further extensive investigations failed to uncover the cause. Her prognosis remained uncertain.

An attempt to wean the child off the ventilator at the university hospital failed, and within a month of her admission she had a tracheostomy. She remained on a ventilator for the rest of her life. Her peripheral weakness became progressively worse, and by 15 months all she could do was shrug her shoulders and slightly flex her right elbow. A sural nerve biopsy showed clear evidence of demyelination. There were abnormal Schwann cells and no significant "onion bulb" formation. This and other evidence led to a diagnosis of autosomal recessive hereditary motor and sensory neuropathy type III with amyelination (HMSN type III). Her condition was unlikely to improve. The hereditary aspects of the diagnosis were discussed with the family.

By now the child was in an adult intensive care unit in a hospital near to her mother's family and a decision had to be made about her further care. She was paralysed but fully alert and had normal cognitive development at 18 months. She could communicate well through her limited gestures, and when presented with computer keys that she could manipulate with her extremely limited movements she could play simple games. She was clearly out of place on an adult intensive care unit.

At a case conference that included her parents, senior staff of the intensive care unit, community and acute paediatricians, paediatric nurses, a child development team nurse and physiotherapist, a paediatric social worker, the business manager of the acute unit (not a trust at the time), the contracts manager of the health authority, and the director of nursing services of the community unit it was decided that the child should be cared for at home. It was calculated that this exercise would cost over £100 000 per year, and funding was sought from the health authority, which agreed to meet the cost. After detailed discussions and preparations she went home in mid-January 1994, aged 2 years.

She lived in a small farmhouse near a village with her mother, brother, and sister aged 5 and 4 and a team of community nurses specially recruited for the task. The nurses had been trained by the staff of the intensive care unit, and family doctors took care of her medical needs with occasional visits from the acute and community paediatricians. Few serious problems occurred during the 16 months she was at home. She had to be admitted twice, once a month after her discharge home and once eight months later because of chest infection with episodes of bradycardia and difficulties with her ventilation. She had recurrent urinary tract infections, painless to her, which were treated by her general practitioner. One of the nurses developed open pulmonary tuberculosis, and the child developed primary chest tuberculosis, requiring isoniazid.

At home she could play games, go for walks in a special perambulator, sit in the garden, interact with her siblings and friends, listen to stories, and watch television. She loved being tickled, was affectionate and frequently cuddled her family.

In May 1995, 16 months after going home and at the age of 3 years and 4 months, she began to suffer increasing episodes of hypoxia and bradycardia. She was given oral antibiotics but quickly deteriorated and lapsed into a coma. Further treatment was not thought

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appropriate by either her family or her doctors and she died peacefully with her mother at home.

Comment

I came to the first case conference sceptical about the value of home care for this child. The cost seemed enormous, and so it proved—something like £160 000. Contemplating the alternatives, however, made care at home seem the right option. One alternative, technically, would have been to allow her to die quickly. This would have been morally wrong and practically impossible. She was alert and felt well: she simply could not breathe for herself. Her cognitive development was normal, and while an attitude of benign (so called) neglect may have been possible during the acute phase of her illness it was out of the question by the time the case conference was held. Even in the early stages her doctors knew neither the diagnosis nor the prognosis of her illness, so such a course of action wasn't possible.

The second option was to leave her in the adult intensive care unit. There she was well looked after and would probably have survived longer than she did. It seemed very unlikely that she had a long term future wherever she was nursed. We felt, however, that it would be awful for her to spend her remaining time in such unnatural surroundings. The third option was to equip and staff a special room in the children's ward for her. This would have cost almost as much as home care without much of the benefit.

So, after careful thought at the case conference, we felt that if we were to treat her at all we had to give her as normal a life as possible for the rest of her probably short life, and that meant ventilation at home, whatever the cost. All contributors to the case conference in September 1993 and all the professionals involved at earlier stages came to the same conclusion.

You may read other arguments about how the money might have been better spent, relieving a greater quantity of misery. I don't believe you can measure misery or the relief of misery, even approximately. Some things are not measurable. The home treatment of this child on a ventilator in order to give her some experience of near normal family life was a costly but beautiful deed and not susceptible to financial analysis or even one using quality adjusted life years (QALY).[1] I applaud the decision of the health authority in this case and I hope that the multidisciplinary committee that now advises the authority on costly cases is capable of such wisdom.

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Commentary: Reach beyond metaphor to assess value

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The case of this child could, like that of child B, have easily developed into a contested issue. The prognoses were equally dismal and the projected cost of child B's transplant was actually less (£75 000)[1] than that of one year's home ventilation. Yet the purchaser's agreement to fund the child's care should not be taken to mean that there are no thorny issues.

Firstly, let me deal with matters of fact. The cost of home ventilation for this child is estimated at £160 000. Was this greater or less than the cost had she remained in hospital? Published reports on this subject, mainly from the USA, generally assert that costs to health care purchasers are lower at home,[2] but this is critically dependent on who delivers home care. Substantial input from qualified nurses can raise home ventilation costs to hospital levels.[3] Furthermore, even if we assume that home care costs no more than hospital care, for the purchasing authority the home care expenditure still adds to the (presumably) unchanged expenditure on the local provider's intensive care unit.

Matters of resource use bring us to some of the key phrases used in this case study. Is it true, for example, that misery cannot be measured, even approximately? Was home ventilation the right course of action to follow "whatever the cost"? Despite Dr Davies's evident distrust of attempts to "measure misery" or use a quality adjusted life year approach to allocating resources, related ideas actually seem to inform his own judgments. How else, for example, could he conclude that this child was happier at home than in hospital other than by judging (or measuring) on an ordinal scale her level of misery? He intuitively generated his own informal "QALY" by trading improved quality of life at home against probable longer survival in hospital and found in favour of home care. And is there really no cost which would dwarf the reduction in misery obtained by home transfer? Would the health authority's total annual budget have been an acceptable price to pay? If the answer is no, then clearly the phrase "whatever the cost" is not to be taken literally and there must exist a "point of indifference" at which cost and benefit are in balance. This is not pedantry—it is important to reach beyond the language of metaphor to identify underlying, and sometimes paradoxical, ideas.

"Is there really no cost which would dwarf the reduction in misery?"

There is a growing international acceptance that health care resources—however generated—will be inadequate, forcing choices to be made between competing therapies.[4] Public health physicians working in purchasing authorities daily face dilemmas in essence no less difficult than those described in this case—with the key difference that they must choose between groups of individuals, rather than individuals themselves. There is much current debate on whether rationing should be carried out explicitly,[5] either under the auspices of a national forum or under local control (such as the committee on costly cases mentioned here) or implicitly.[6]

Public consultation exercises suggest that there is strongest support among the general population for expenditure on treatments for children (such as this child) with life threatening illnesses, with lowest priority attached to expenditure on those over 75 years.[7] If such public consultation becomes more common—and if notice is taken of its findings—the clinical action followed here may actually receive explicit popular encouragement. Conceivably British paediatricians could even find themselves having to defend their more conservative decisions not to ventilate children. An example is children with Duchenne muscular dystrophy, who have not traditionally been considered candidates for ventilation in the UK but who are in the USA.[8] What, it might reasonably be asked, is the material difference between a child with polyneuropathy in respiratory failure and one with muscular degeneration in respiratory failure? They might well be judged equally worthy of this "costly but beautiful deed." If they are not so judged then the reasons may need to be made explicit.

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