Prognosis for survival and improvement in function in children with severe developmental disabilities

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Objective: To derive prognostic data for survival and clinical improvement in children with severe developmental disabilities.

Study design: A 13-year follow-up study of several cohorts of children initially evaluated before their first birthday. The outcomes studied were survival and improvement in condition. Methods were used to overcome limitations in previously published work on the same California data base. Of the 11,912 children who received services from the California Department of Developmental Services between January 1980 and December 1993, we focused on three cohorts defined according to mobility and need for tube feeding.

Results: Children who were tube fed and unable to lift their heads by ages 3 to 12 months were at high risk for early death, with a median remaining life expectancy of 3.2 years. Of those who survived an additional 2 years, the condition of about one third improved. A substantial majority of those who either showed improvement or died had done so by that age.

Conclusion: By age 5 years, the prognoses for survival and improvement have to a large extent been clarified. For children who survive to age 5 years, even those in the lowest functioning cohort have a 60% chance of surviving an additional 5 years. Detailing the probabilities of various outcomes at various ages should be useful to parents, pediatricians, and others concerned with children with developmental disabilities. (J Pediatr 1997;131:712-17)

Children with severe developmental disabilities are subject to a high mortality rate.[1-8] With the trend towards deinstitutionalization,[9-11] even severely disabled children now usually live in their own homes or in community care facilities rather than in state institutions. This has created a challenge for pediatricians and other caregivers in the community, many of whom have not worked extensively with developmentally disabled children. Data on prognosis for survival and improvement in this population should be helpful to parents, physicians, and other service providers.

The most powerful predictors of an unfavorable outcome are known to be various measures of immobility, together with the need for tube feeding.[7,8] A number of earlier studies reported on the survival of children with disabilities.[1-8] Eyman et al.[7] reported on the life expectancy and survival prospects of six subgroups of persons with severe disabilities whose conditions *remained static* over time. Because children whose conditions subsequently improved were excluded, life expectancies were shorter than would have resulted if a cohort of all children *initially* in the subgroup had been followed up. The distinction between the two approaches has sometimes been misunderstood by users and deserves emphasis.

CDER Client Development Evaluation Reports

In this study we worked almost entirely from a cohort perspective. This is appropriate if a prognosis for a child currently in a given condition is required, because it will not yet be known whether the child will remain in the initial condition. Also, only children evaluated within their first year were included, so that their developmental history was well determined. Further, we were able to use 3 additional years of follow-up evaluation and to exploit a more refined classification system of predictor variables that was identified in subsequent work.[8] We focused on several cohorts defined on the basis of the early level of functioning of the child and need for tube feeding. The main questions were as follows:

1. At an early age, what are the prognoses for survival and improvement for each of these cohorts?
2. What is the age range at which the ultimate outcome is best predicted?
3. What is the prognosis for survival once a child has reached this age range?

METHODS

Instrument and Variables

All our information on the characteristics of children in this study was derived from the Client Development Evaluation

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Reports of the subjects.[12] A requirement for reimbursement of services is that a CDER be submitted annually to the California Department of Development Services, and when a person with mental retardation moves to a new placement. The CDER includes a 66-item measure of adaptive behavior. The inter-rater reliability of the items used has been investigated elsewhere[13,14] and judged to be good. All deaths that occurred between January 1980 and December 1993 were included in our analysis. Deaths are reported to the Department of Development Services by the 21 regional centers in California. We also incorporated mortality data from annual tapes issued by the California Bureau of Vital Statistics; causes of death were determined by the ICD-9 codes from this source.

The primary study variables comprised the following:

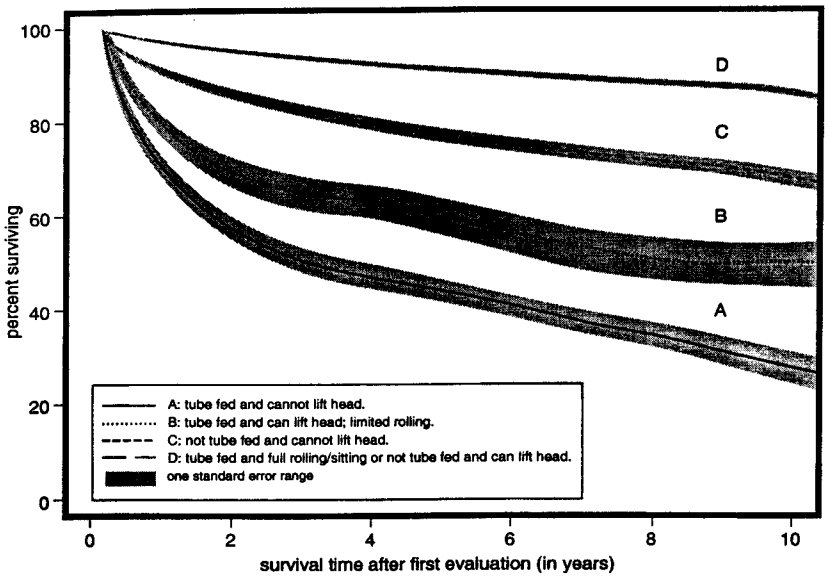

Fig. 1. Survival times subsequent to evaluation for four cohorts of children with developmental disability who are evaluated before age 1 year. Five-year survival probabilities: group A, 43%; group B, 62%; group C, 77%; group D, 91%. Median survival times: group A, 3.2 years; group B, 11.5 years; median survival times for groups C and D exceed study period and cannot be computed.

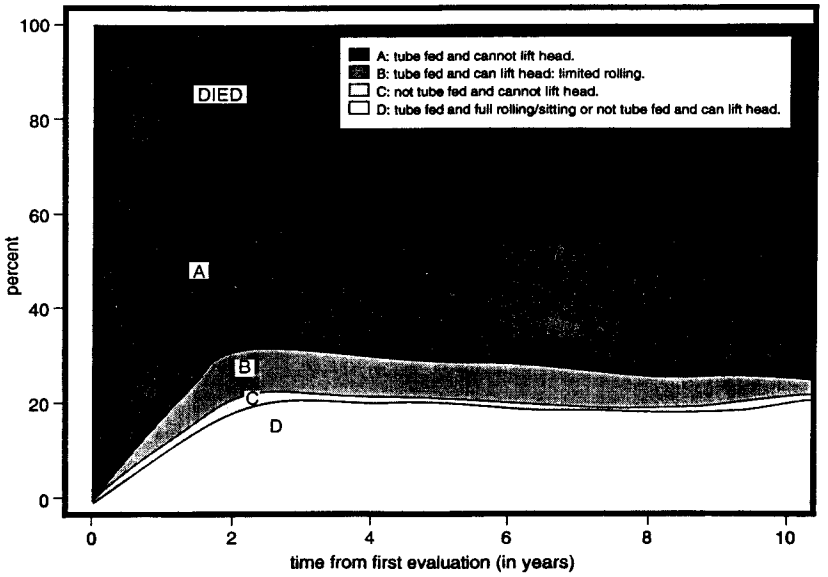

Fig. 2. Probabilities of the condition showing subsequent improvement and of remaining static for children initially in group A. Results apply to a cohort not subject to censoring, so that all surviving members must be in one of the four groups A to D.

ROLLING AND SITTING

Of the 66 evaluation items on the CDER, we used Rolling and Sitting (item no. 1) as a primary classification variable. This is a nine-point scale ranging from "Cannot lift head when lying on stomach" to "Sits independently without support for 5 minutes." On the basis of previous mortality analysis,[8] this variable was collapsed to the following four-point scale:

1. Cannot lift head when lying on stomach.
2. Can lift head but not chest when lying on stomach.
3. Can lift head and chest when lying on stomach but has at most partial ability to roll. (Specifically the child does not have ability to roll both from front to back and back to front).
4. Has full rolling ability or has ability to sit with or without support.

The inter-rater reliability of this four-point scale as assessed by Cohen's weighted kappa[15] was 0.90.

TUBE FEEDING

Feeding by means of a nasogastric or gastrostomy tube was provided for children who had impaired swallowing or who frequently aspirated food. Although the CDER does not fully specify the type of feeding tube, the great majority of these children were believed to have been fed with gastrostomy tubes.

Subjects

Of the population of 177,500 persons who received services for developmental disabilities in California during the study period, we studied the 11,912 children who were evaluated on or before their first birthday. The majority of children with severe neurologic disorders are known to be evaluated by this age. The children whose first evaluation occurred after their first birthday were excluded. Evaluations performed at age less than 3 months were also excluded, because infants of that age have a limited repertoire of motor activities, and we did not believe

Table. Characteristics of the four groups of children with developmental disabilities

	Group				
	A	B	C	D	All
Sample size	553	296	1,849	9,214	11,912
Level of MR*					
Mild	3.4	7.1	8.2	8.8	8.4
Moderate	3.1	2.7	3.6	3.3	3.3
Severe/profound/suspected	93.5	90.2	88.2	87.9	88.3
Rolling and sitting					
Doesn't lift head	100.0	0.0	100.0	0.0	20.2
Lifts head	0.0	67.6	0.0	33.2	27.3
Lifts head and chest	0.0	12.8	0.0	10.8	8.7
Rolls side to side	0.0	11.1	0.0	7.8	6.3
Rolls front to back	0.0	8.4	0.0	9.9	7.8
Rolls over	0.0	0.0	0.0	16.9	13.0
Sits for 5 min. w/support	0.0	0.0	0.0	8.2	6.3
Sits for 5 min w/o support	0.0	0.0	0.0	5.6	4.3
Sits independently	0.0	0.0	0.0	7.7	6.0
Hand use					
No functional use	82.6	37.5	70.5	22.0	32.7
Partial use	16.5	55.4	26.4	57.7	50.8
Full use	0.5	4.1	1.4	11.0	8.8
Unknown	0.4	3.0	1.7	9.4	7.6
Arm use					
No functional use	72.9	23.3	54.5	11.5	21.3
Partial use	10.1	23.6	15.0	14.6	14.6
Full extension	8.0	20.6	13.8	21.8	19.9
Unknown	9.0	32.4	16.7	52.1	44.1
Tube feeding					
Yes	100.0	100.0	0.0	0.7	7.7
No	0.0	0.0	100.0	99.3	92.3
Residence					
Own home	59.9	80.1	91.6	94.7	92.2
Community care	6.7	5.7	5.0	4.2	4.5
Intermediate care facility	2.7	1.0	0.5	0.1	0.3
Institution	17.7	7.1	0.8	0.1	1.2
Skilled nursing facility	4.7	1.0	0.7	0.2	0.5
Other	8.3	5.1	1.5	0.7	1.3

All subjects were evaluated before the age of 1 year. Group A was tube fed and unable to lift head when prone. Group B was tube fed and able to lift head, but with at most partial rolling ability. Group C was not tube fed but unable to lift head when prone. For comparison, group D is included in the study; it consists of all the other children evaluated before age 1 year. Except for sample sizes, all figures are percentages.

*Grossman HJ, editor. Classification in mental retardation. Washington, DC: American Association on Mental Deficiency; 1983. p. 184. Levels are Mild (IQ from 50 or 55 to approximately 70), Moderate (IQ from 35 or 40 to 50 or 55), Severe (20 or 25 to 35 or 40), and Profound (below 20 or 25). California uses an additional category, "suspected," for those whose retardation has not been formally tested; this group is subject to mortality rates comparable with those in the severe and profound groups.

that mobility-related CDER items could serve as a valid prognostic indicator in this age group. Thus our sample consisted of children who received a CDER evaluation between 3 and 12 months of age. Because of the high costs of care for children with severe developmental disabilities, it is believed that the great majority are served by the state system and thus were included in our population. We were able to obtain some causative data from the CDER. The major groups were as follows: chromosomal anomalies, 26%; infections, 19%; prenatal or perinatal insults, 15%. Most of the remaining 40% had an unknown cause. Our interest centered on three nonoverlapping subgroups, A, B, and C with severe disabilities, and a fourth group, D, that served as a control group. Subjects in group A (n = 553) were tube fed and unable to lift their heads when lying on their stomachs (level 1 of the four-point Rolling and Sitting variable). Subjects in group B (n = 296) were tube fed and able to lift their heads but lacked full ability to roll (levels 2 or 3). Subjects in group C (n = 1849) were not tube fed but were unable to lift their heads (level 1). Subjects in group D (n = 9214) were all other children evaluated before their first birthdays (i.e., either tube fed with full rolling ability or not tube fed but able to lift heads).

The reduction of the eight possible combinations of mobility levels and presence/absence of tube feeding to the four groups A, B, C, D was carried out on the basis of preliminary analyses showing these groups to be homogeneous with respect to subsequent survival.

Statistical Analysis

Analyses were carried out by using SAS (Statistical Analysis Systems, SAS Institute, Inc., Cary, N.C.), and graphs were constructed by using the package S-PLUS. For the basic survival analyses, the Kaplan-Meier product-limit survival method[16] was used. The log-rank test[17] was used for formal comparisons of cohort-specific differences in the survival curves. Standard errors and confidence intervals for survival times were based on the Greenwood formula.[16]

Data were expressed as median survival times (time at which only 50% of the cohort still survive) rather than life expectancies[17] (*mean* or *average* of the survival times). The life expectancy is more influenced by unusually large observation populations than is the median survival time and is thus generally the longer of the two. Life expectancies cannot be computed from a survival analysis unless the uncensored survival time of all individuals is known, which is

rarely the case for a real cohort. In some instances fewer than 50% of a cohort died before the end of the study period, so that median survival times could not be reported. For these cases the reported 5-year and other survival probabilities may be used as summary statistics. Because children who died before an evaluation could be made would not be in our sample, the data were subject to "left truncation."[18]

Results on prognosis for improvement apply to a hypothetical cohort not subject to censoring or live withdrawal. There were two stages in the computations. We first obtained Kaplan-Meier estimates[17] of the probability that the subject was alive at each age, given that he or she was not censored or withdrawn. Second, of the children alive and uncensored at each age, the proportions in groups A, B, C, or D were computed. This procedure can be shown to yield maximum likelihood estimates of the chances of being in these groups, conditional on the subject being alive and uncensored.[19]

RESULTS

The Table gives descriptive statistics on the four groups. Group A is the most debilitated, followed by groups B, C, and D. For example, children in group A were more likely to have restricted hand and arm use, have more severe degrees of mental retardation, and were also more likely to reside in an institutional setting rather than in their own home.

Survival of the Initial Cohorts

Fig. 1 shows the survival curves for the four groups, with survival times beginning at the age of CDER evaluation. As expected, group A had the poorest survival, with a median survival time of only 3.2 years. Ten years after evaluation, only 27% had survived. The median survival time for children in group B, who were tube fed but had limited mobility, was appreciably longer (11.5 years). Groups C and D had a better prognosis; median survival times exceeded the study period and could not be computed, but the 10-year survival probabilities were 68% and 86%, respectively.

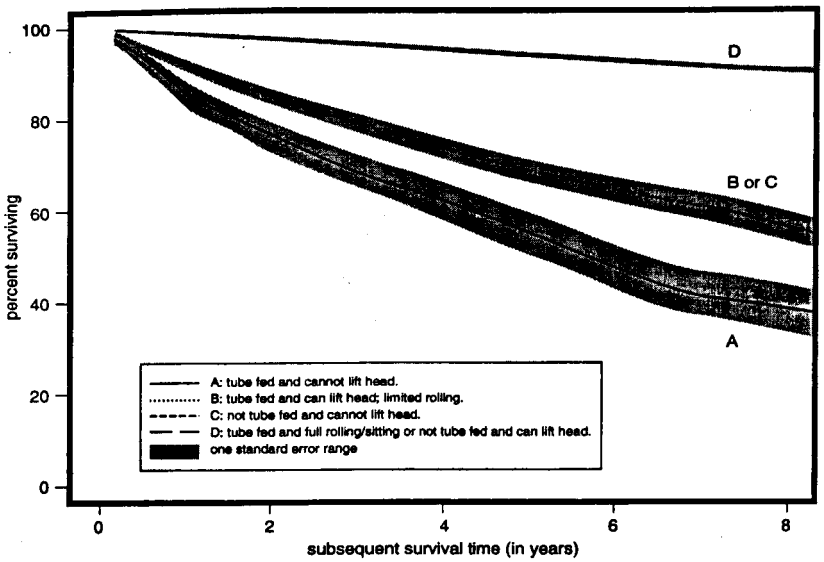

Fig. 3. Additional survival for children aged 2 to 6 years, grouped according to current status. Groups B and C have been combined. Median survival for group A: 5.7 additional years; for groups B and C, 9 additional years (estimated by extrapolation). Five-year survival probabilities: group A, 55%; group B, 69%; group C, 69%; group D, 94%.

Prognosis for Improvement

Fig. 2, which depicts the 10-year follow-up data for group A, corresponds to a hypothetical population not subject to censoring or other withdrawal from the study. Thus patients either remain in group A, die, or transfer to one of the other groups. The data presented track the percentage of patients for each of these possible outcomes during the follow-up period. For example, 100% of the cohort were in condition A at time t = 0. Two years later, more than 40% had died and more than 30% had shown improvement. The majority of the children whose condition had improved had changed to condition D, the best-functioning level. After this 2-year period, the chances of improvement are poor, with death becoming a much more likely outcome than improvement. Thus most of the children whose condition would improve would have done so before age 3 years.

For group B (not shown), 35% had died and 41% had improved to condition D after 4 years. By age 5 years, most of those whose condition would improve or who would die had already done so. For group C (not shown), those who improved (about two thirds of the cohort) had mainly done so by age 3 years. For both groups B and C, deterioration to a worse condition (e.g., B or C to A) occurred infrequently. The same was true for group D (not shown).

Effect of Initial Cohort Membership on Prognosis, Given Current Age and Group Membership

Given the current age and group membership of a child (A, B, C, or D), we investigated whether subsequent survival was affected by the cohort to which the child initially belonged. No systematic trends were observed, and differences in all cases were statistically insignificant at the 5% level.

Survival Subsequent to Age 2 Years

As previously noted, the 5-year survival probabilities for the four initial cohorts in the first year of life were as follows: group A, 43%; group B, 62%; group C, 77%; and group D, 91%. Fig. 3 shows the calculated additional survival times for the four groups for the children who had survived to age 2 to 6 years. Initial analysis showed that for each cohort group the additional survival times were effectively the same for children when examined separately at ages 2, 3, 4, 5, or 6 years. We thus combined the data for these age groups for each of the four cohorts. Further, groups B and C were combined because

their survival curves were very similar. The 5-year survival rates for the groups were as follows: A, 55%; B or C, 69%; and D, 94%. The median survival time for group A was 5.7 additional years; for the other groups the median could not be computed.

DISCUSSION

This large-scale epidemiologic study tracked survival and clinical improvement of cohorts of infants with developmental disabilities. A significant percentage of severely impaired children acquire certain motor functions that are associated with improved long-term survival. This usually occurs by ages 3 to 5 years, depending in part on the initial severity of the disability. After that, the chances for functional improvement and a corresponding increase in survival prospects are substantially diminished. The sharp differences seen between the four cohorts strongly suggest that variables that are clinically simple to assess (i.e., tube feeding, ability to lift head, and ability to roll over) are powerful predictors of survival and potential for improvement. As with any such work, the findings apply to conditions that were obtained during the study period (here, 1980 to 1993), and current and future changes in medical practice may result in changes in prognosis. Nevertheless, the figures documenting chances of survival and improvement at various ages may provide a helpful summary of our current knowledge.

Our results may be contrasted with those of Eyman et al.,[2,7] who reported survival probabilities and related quantities for children whose condition remained static. It should be emphasized that, unlike those studies, the current analyses may be used for the prognoses of children currently in a specified condition. The approach of Eyman et al.[2,7] resulted in lower survival probabilities than those reported here because children whose condition improved were excluded. For example, we calculated that their survival analysis would result in a median survival time for our group A of only 9 months, compared with the 3.2 years in our analysis.

As noted, we found that among children of a given age and current group membership (A, B, C, or D), the subsequent survival chances did not depend on the initial cohort membership of the child. Many patients do not see the same physician over long periods. Our finding suggests that physicians may make some prognoses for children aged 2 to 6 years, irrespective of conditions present during infancy. It should be cautioned, however, that more complete historic information could affect the subsequent prognosis. For example, the number of times that the child had been hospitalized might be of predictive value, even controlling for current status.

The striking improvement noted in survival of group B compared with that of group A is the result of many factors. Infants who are able to lift their heads (group B) are better able to handle secretions, have a reduced incidence of both chronic microaspiration and massive aspiration, and have fewer secondary bacterial infections. Such infants also have less spasticity and posturing and thus are less prone to airway compromise, obstructive and central apnea, and intermittent and chronic hypoxia, factors that contribute to sudden and unexpected deaths in this population.

The differences in survival between group C and group A are even more striking. Children in the former group, although unable to lift their heads, are able to be fed and to swallow, and thus have more coordinated oropharyngeal and esophageal reflexes than the group A infants. Although the group B infants were able to overcome the effects of gravity or stasis on food and secretion movement by lifting their heads and/or rolling, the group C infants could actively suck, chew, and swallow their food and propel it to the stomach. This would suggest that the incidence of aspiration in group C might be lower than in groups A or B and might explain the lower mortality rate. Group C infants may have had a lower incidence of obstructive and central apnea, which presumably reflects more intact brain-stem function and contributes to their increased survival.

Estimation of the life expectancy of children with severe developmental disabilities can be made on the basis of serial observations of relatively basic motor skills. Irrespective of cause, a proportion of such children, examined within the first year of life, will show clinical improvement and with that, have a longer life expectancy. Thus an initial determination of life expectancy may need revision at age 3 to 5 years; however, beyond that time, chances for continued neurologic improvement that would affect estimations of life expectancy are unlikely.

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