primary studies - published RCT

Early diagnosis of cystic fibrosis through neonatal screening prevents severe malnutrition and improves long-term growth. Wisconsin Cystic Fibrosis Neonatal Screening Study Group.

Code: PM11134427 **Year:** 2001 **Date:** 2001 **Author:** Farrell PM

Study design (if review, criteria of inclusion for studies)

randomized clinical trial from 1985 to 1991 (dried newborn blood) and from 1991 to 1994 (DNA-based detection of the DeltaF508 mutation).

Participants

n= 650 341 newborns. 2 groups-an early diagnosis, screened cohort and a standard diagnosis or control group.

Interventions

Wisconsin CF Neonatal Screening Project

Outcome measures

benefits and risks of early diagnosis through screening. In addition, the incidence of CF was determined, and the validity of our randomization method assessed by comparing 16 demographic variables. statistical analysis of anthropometric evaluated indices that includes all CF patients without meconium ileus.

Main results

The incidence of classical CF, ie, patients diagnosed in this trial with a sweat chloride of 60 mEq/L greater, was 1:4189. By incorporating other CF patients born during the randomization period, including 2 autopsy diagnosed patients and 8 probable patients, we calculate a maximum incidence of 1:3938 (95% confidence interval: 3402-4611). Although there were group differences in the proportion of patients with DeltaF508 genotypes and with pancreatic insufficiency, validity of the randomization plan was demonstrated by analyzing 16 demographic variables and finding no significant difference after adjustment for multiple comparisons. Focusing on patients without meconium ileus, we found a marked difference in the mean +/- standard deviation age of diagnosis for screened patients (13 +/- 37 weeks), compared with the standard diagnosis group (100 +/- 117). Anthropometric indices of nutritional status were significantly higher at diagnosis in the screened group, including length/height, weight, and head circumference. During 13 years of study, despite similar nutritional therapy and the inherently better pancreatic status of the control group, analysis of nutritional outcomes revealed significantly greater growth associated with early diagnosis. Most impressively, the screened group had a much lower proportion of patients with weight and height data below the 10th percentile throughout childhood.

Authors' conclusions

Although the screened group had a higher proportion of patients with pancreatic insufficiency, their growth indices were significantly better than those of the control group during the 13-year follow-up evaluation and, therefore, this randomized clinical trial of early CF diagnosis must be interpreted as unequivocally positive. Our conclusions did not change when the height and weight data before 4 years of age for the controls detected by unblinding were included in the analysis. Also, comparison of growth outcomes after 4 years of age in all subjects showed persistence of the significant differences. Therefore, selection bias has been eliminated as a potential explanation. In addition, the results show that severe malnutrition persists after delayed diagnosis of CF and that catch-up may not be possible. We conclude that early diagnosis of CF through neonatal screening combined with aggressive nutritional therapy can result

http://www.ncbi.nlm.nih.gov/pubmed/11134427

See also

Pediatrics. 2001 Jan;107(1):1-13.

Keywords

Failure to Thrive; Food; Neonatal Screening; Newborn; non pharmacological intervention - diagn; non pharmacological intervention - diet; Nutrition Disorders; prevention; screening; diagnostic procedures;