

Presenting Symptoms of Cystic Fibrosis and Long-Term Survival

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The Northern New England Cystic Fibrosis Consortium



The NNECFC is a regional, voluntary consortium of more than 80 clinicians and researchers from the CF care centers in Maine, New Hampshire and Vermont. The mission of the group is to improve CF care and patient outcomes.

Goal

The presenting symptoms of CF have been well described. The goal of these analyses was to assess the long-term survival outcomes of CF patients by presentation characteristics.

Methods

The CFF Registry contains data on complications at birth, diagnosis suggested by/symptoms at first CF work-up, patient years of follow-up and survival. We selected genotyped patients diagnosed before age 18 years and having one or more records in the Registry since 1991 (n=15,180). Presentation was categorized as asymptomatic, respiratory only, multiple symptoms, meconium ileus. Kaplan-Meier plots were calculated and Cox proportional hazards regression was used to adjust for baseline differences in genotype, sex, age at diagnosis, race/ethnicity.

Incidence of Death Among CF Patients by Presentation per 10,000 yrs of patient followup

Presenting Symptoms	Deaths/10 ⁴ person yrs	Deaths N	Person Years	p value
Asymptomatic*	38.1	72	18,898	reference
Respiratory only	49.8	181	36,380	0.015
Multiple Symptoms	51.1	232	138,090	0.002
Meconium ileus	55.8	706	41,575	0.002

*Asymptomatic was defined as diagnosis based exclusively on one or more of the following: genotype, family history, neonatal screening, prenatal diagnosis (CVS or amniocentesis).

Results

Among these 15,180 patients there were 1191 deaths. The incidence density rates per 10,000 person-years varied widely by presentation: asymptomatic (38.1), respiratory only (49.8), multiple symptoms (51.1), and meconium ileus (55.8). The log rank test was statistically significant. $\chi^2=37.2$, $p<0.0001$.

Further adjustment for patient and disease characteristics did not materially change these results.

Conclusions

- Patient presentation is a statistically significant predictor of long-term survival in CF.
- Meconium ileus, respiratory presentation and multiple symptom presentation have substantially higher mortality rates than do asymptomatic presentation.

Kaplan-Meier Long-term Survival Estimates by Presenting Symptoms

