

Appendix

Methods for Assessing Programs' Pulmonary and Nutritional Outcomes

Outcomes of interest

The disease measures of interest for pediatric programs were FEV₁ percent predicted (calculated using the Knudson equations) and Body Mass Index percentile (BMI%).

Due to the different relationship between age and these two aspects of disease, we created age strata of 6-12 years and 13-17 years to analyze FEV₁ percent predicted, and age strata of 2-11 years and 12-17 years to analyze BMI%. Disease measures of interest for Adult Program analyses were FEV₁% predicted and absolute BMI.

Analysis data set

We evaluated CF registry data for the years 2000-2004 for pediatrics and 2001-2005 for adults. Pediatric Programs were included in the analysis data set if they reported greater than 50 patients in 2004 and at least 10 patients per year in each age stratum (6-12, 13-17, and 2-11 years). Adult Programs were included in the analysis set if they reported at least 40 patients. All patients who had undergone a lung transplant were excluded from the analysis data set.

Univariate analyses

1. FEV₁. The FEV₁ percent predicted was calculated each year for each individual patient by determining the mean of the highest FEV₁ percent predicted reported for that patient for each quarter. A center-level FEV₁ was calculated separately for each age

stratum using the median of the mean annual patient FEV₁ measurements for the years 2000-2004 (pediatrics) and 2001-2005 (adult). These center-level median values were ranked and divided into quintiles.

2. BMI

The BMI% was utilized for pediatric programs and was calculated each year for each individual patient based upon the last height and weight reported during each calendar year. A center-level BMI was calculated separately for each age stratum using the median of the annual patient BMI measures. These center-level median values were ranked and divided into quintiles. Median absolute BMI utilizing all measurements was utilized as primary nutritional outcome measure for adult programs.

Adjustment for Case-Mix Differences Between Centers

We examined the center-based distribution of the following patient characteristics: patient age, gender, pancreatic status (defined by use of enzymes), median income (based on 2000 US Census data), and race/ethnicity. These have been determined in previous work to be to important determinants of disease outcome and therefore potential confounders requiring adjustment when comparing center outcomes.

Multivariable analyses

Multivariable analysis using General Estimating Equations (GEE) was performed with the five year dataset previously described, to predict patient FEV₁ percent predicted or

BMI in relation to the following independent variables: age (as a continuous variable), gender, pancreatic status (defined by use of enzymes), median income (based on 2000 US Census data), race/ethnicity, and center. These variables have been determined in previous work to be to important determinants of disease outcome and therefore potential confounders requiring adjustment when comparing center outcomes. The GEE approach takes into account the strong correlation between yearly patient measures. We then calculated the adjusted average FEV₁ percent predicted and the average BMI by summing the products of the model coefficients times the average value of each of the independent variables for each center and adding the coefficient of each center's indicator variable. In other words,

Adjusted FEV₁ percent predicted at program X =

β_0 (a constant) + $\beta_1 \times$ (average age of patients in stratum of interest at center x) + $\beta_2 \times$ (proportion males) + $\beta_3 \times$ (proportion pancreatic sufficient) + $\beta_4 \times$ (proportion with median family income <\$30K) + $\beta_5 \times$ (proportion Caucasian non-Hispanic) + $\beta_6 \times$ (center x indicator).

Center adjusted data

Adjusted center mean FEV₁ percent predicted values and mean BMI values were divided into quintiles. Centers were eligible to be chosen by pulmonary or nutritional outcomes if they were consistently in the top quintile for an analyzed category.

Adult Program Analysis Corrections

To assure accurate assessment of quality of adult care, we controlled for the effect of pediatric care in years prior to transition by analyzing only patients 25 years or older and adjusting FEV₁ and BMI for the values in those patients 7 years prior.