Characterization and Waiting List Outcomes of Patients With Cystic Fibrosis Listed for Lung Transplantation

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INTRODUCTION

• Cystic fibrosis (CF) is the third most common indication for lung transplantation and is often the only remaining option for patients with CF who have end-stage lung disease.

• While prior studies1 have evaluated post-transplantation outcomes, patients with CF who are waiting for a lung transplant have not been well characterized.

OBJECTIVES

• To characterize patients with CF at the time of listing on the lung transplant waiting list and describe their outcomes while on the waiting list.

• To characterize the subset of those patients who received a lung transplant.

METHODS

• This retrospective cohort study used data from the Scientific Registry of Transplant Recipients (SRTR) Standard Analytic File2 for 2006-2014.

• The year 2006 was chosen as the first full calendar year during which the lung allocation score was implemented, and 2014 was the last calendar year for which data were available at the time this analysis was undertaken.

• The SRTR includes data on all organ donors, waiting list candidates, and transplant recipients in the United States.

• This study included only candidates and recipients who were listed on the lung transplant waiting list as of January 1 of each year and who had a primary diagnosis of CF.

Characterization of the Overall Lung Transplant Waiting List for Patients With CF (2006-2014)

• To characterize the overall lung transplant waiting list for patients with CF: the waiting list was examined for each calendar year between 2006 and 2014.

• For each year, the number of patients who were summarized who were:

  – On the waiting list as of January 1 of the relevant year

  – Listed for lung transplantation during the relevant year, including patients who had a previous lung transplant or previous listing

• Patients were only counted once per year but could be included in multiple years.

• Among all patients included within a given year (those on the waiting list as of January 1 and those listed during the year), the waiting list outcome recorded within that year for each patient was summarized. The outcomes were:

  – Received lung transplant

  – Died

  – Removed from list for other reasons.

• Patients who remained on the list as of December 31 of that year were categorized as still waiting.

Candidate and Waiting List Outcomes Analyses Among Newly Listed Patients

• The cohort used for these analyses (the ‘recipient cohort’) included patients with CF who were added to the waiting list during the waiting list period from 2006 to 2014, who had no previous lung or heart-lung transplant history, and no pre-existing lung disease.

• Demographic and Clinical Characteristics

• Demographic and clinical characteristics for the candidate cohort were summarized overall and stratified by age at listing(12-17 vs >18).

• Functional status was reported using the Kamofsky performance score for adult patients and the Lansky performance score for pediatric patients. This metric is reported in 10% increments between 10% and 100%.

• Patients were grouped into 3 categories of functional status restrictions as defined by their Kamofsky or Lansky performance score:

  – Category 1: Patients were severely limited in reactivity (10–30%)

  – Category 2: Patients were moderately limited in reactivity (30–70%)

  – Category 3: Patients had normal reactivity (70–100%)

• Counts and percentages were used for categorical variables, and mean and standard deviation (SD) and median (interquartile range [IQR]) were used for continuous variables.

• Result Interpretation:

  – Patients from the candidate cohort were followed from listing date until the earliest of removal from the waiting list (any reason) or the date 2 years after listing. Outcomes were collected 1, 3, 6, 12, and 24 months postlisting.

• Five outcome measures were used for each: patients who received lung transplants; those who died; those who became ineligible due to deteriorating health; those who were no longer listed as of listing date; and those who died before listing.

RESULTS

Summary of the Overall Lung Transplant Waiting List for Patients With CF (2006-2014)

• From 2006-2014, the number of patients with CF who were on the lung transplant waiting list as of January 1 of each year appeared to decline, then remained stable through 2014.

• The number of patients who received a lung transplant appeared to remain relatively stable between 2006 and 2014 (range 179-243) (Table 1).

• The median time to lung transplantation was 3 months (IQR: 1-7) among those who received a lung transplant. 10% who did not survive (Figure 1) and 5% who became ineligible due to disease progression (Figure 2) were removed from the list due to improved health.

• The median time to lung transplantation was 24 months (IQR: 12-36) among those who received a lung transplant, 24% waited ≥6 months (Waiting List Outcome Analyses: Subgroup 2015). 5% who became ineligible due to disease progression (Figure 2) were removed from the list due to improved health.

CONCLUSIONS

• The disease state among patients with CF on the lung transplant waiting list was severe, as reflected in reduced lung function, functional limitations, presence of comorbidities and complications, and use of life support.

• Once listed for lung transplantation, few patients were removed from the list due to improved health.

• While many patients received a lung transplant within 24 months, nearly 1 in 10 patients waited ≥6 months for a lung transplant, and 10% did not survive.

• Given the severity of disease among these patients and the fact that not all may receive a transplant once listed, maintaining health and delaying disease progression is critical in patients with CF.

REFERENCES


AUTHOR DISCLOSURES

LG, EK, and MW are employees of Vertex Pharmaceuticals Incorporated and may have stock or stock options in that company. Medical writing support was provided by Mary Kacillas, JD, and may own stock or stock options in that company. Medical writing support was provided by Mary Kacillas, JD, and may own stock or stock options in that company.

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