

44th Annual Conference of the International Society for Clinical Biostatistics - Joint conference with the Italian Region of the International Biometric Society

University of Milano-Bicocca

27/08/23 - 31/08/23

Not definitive text format

Document ID: 109142

Authors: Simone Gambazza Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Healthcare Professions Department ~ Milano ~ Italy
Annalisa Orenti University of Milan, Department of Clinical Sciences and Community Health, Laboratory of Medical Statistics, Biometry and Epidemiology "G. A. Maccacaro" ~ Milano ~ Italy
Anna Zolin University of Milan, Department of Clinical Sciences and Community Health, Laboratory of Medical Statistics, Biometry and Epidemiology "G. A. Maccacaro" ~ Milano ~ Italy
Federico Ambrogi University of Milan, Department of Clinical Sciences and Community Health, Laboratory of Medical Statistics, Biometry and Epidemiology "G. A. Maccacaro" ~ Milano ~ Italy

Topic: Competing risks and multistate models

Abstract Title: A MULTI-STATE MODEL EVALUATING THE ASSOCIATION OF OXYGEN THERAPY WITH THE COURSE OF CYSTIC FIBROSIS IN EUROPE

Introduction and Objective(s): The most serious complications of cystic fibrosis (CF) relate to respiratory failure, leading to hypoxemia, which is the time when the respiratory system is profoundly compromised by the disease. Oxygen therapy (OT) is then prescribed to restore oxygen levels in the blood. [1] Also, some people with CF (pwCF) can have lung transplantation (LTx) during their life. Association between dependence OT and natural disease progression in pwCF becomes challenging, and it has not been estimated yet. We therefore used the multi-state model to estimate the transition probabilities from being alive without LTx to LTx and to death, and from being alive after LTx to death in pwCF with and without OT.

Method(s) and Results: We used 10 years' data from the 35-country European CF Society Patient Registry (ECFSPR). A multi-state regression model was fitted using age as timescale to assess the effects of individual risk factors on transition probabilities. We considered 48,343 pwCF aged 6 to 50 years. OT (HR 5.78, 95%CI: 5.32 - 6.29) and abnormal FEV1 (HR 6.41, 95%CI: 5.28 - 7.79) were strongly associated with the probability of having LTx; chronic infection with *Burkholderia cepacia* complex (HR 3.19, 95%CI: 2.78 - 3.67), abnormal FEV1 (HR 5.00, 95%CI: 4.11 - 6.08) and the need for OT (HR 4.32, 95%CI: 3.93 - 4.76) showed the greatest association with the probability of dying without LTx. Once pwCF received LTx, OT (HR 1.75, 95%CI: 1.41 - 2.16) and abnormal FEV1 (HR 1.63, 95%CI: 1.18 - 2.25) were the main factors associated with the probability of dying. We also found an association between gross national income and the probability of receiving LTx, which is lower for pwCF living in low-income European countries.

Conclusions: Oxygen therapy, as a proxy for disease severity, is associated with poor survival in pwCF, even after LTx.; harmonization of CF care throughout European countries remains of paramount importance.

References: [1] Elphick HE, Mallory G. Oxygen therapy for cystic fibrosis. *Cochrane Database Syst Rev.* 2009; (1):CD003884. doi:10.1002/14651858.CD003884.pub3

Keywords: Multi-state model, cystic fibrosis, survival analysis

Status: Ready to send

Preferred presentation type: Poster

Print