Extended Abstract

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A Novel Tool to Evaluate the Accuracy of Predicting Survival and Guiding Lung Transplantation in Cystic Fibrosis

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Abstract

Effective transplantation recommendations in cystic fibrosis (CF) require accurate survival predictions, so that high-risk patients may be prioritized for transplantation. In practice, decisions about transplantation are made dynamically, using routinely updated assessments. We present a novel tool for evaluating risk prediction models that, unlike traditional methods, captures classification accuracy in identifying high-risk patients in a dynamic fashion.

Keywords

Cystic fibrosis; Lung transplantation; Survival; Risk prediction; Classification accuracy.

Introduction

Lung transplantation has been shown to improve survival for some cystic fibrosis (CF) patients whose disease is no longer amenable to more conventional medical therapies [1,2]. However, due to a shortage of donor lungs, a large number of wait-list patients die while awaiting transplantation employing the current allocation system in the US. In 2010-2012, the wait-list deathrate was 15.4 per 100 wait-list years. Candidates aged 12-17 years had the very best wait-list mortality, at 19.7 deaths per 100 wait-list years, followed by those aged 18-34 years at approximately 18.5 deaths per 100 wait-list years [1]. Despite a rise within the rate of lung transplants over the past decade, wait-list mortality rates still rise.

Accurate predictions of mortality are necessary so that limited donor lungs may be prioritized to patients who are at the greatest risk of death without transplantation. The goal is to use a patient's clinical characteristics to calculate the predicted risk of mortality within a specified time period and to rank or classify patients on the wait-list as those that are predicted to die soon versus those that aren't.

Discussion

In the CF setting, we found that updated measurements of FEV1% have consistent performance over time, whereas the performance of a baseline measurement declines over time. Thus, previously reported estimates of the accuracy of FEV1% alone don't capture its true performance during a clinical setting. It is clear that patient information should be updated over time to take care of classification accuracy; however, it's also evident that neither FEV1% alone nor existing multivariate models are adequate for use in practice.

Being able to guage a model's time-varying accuracy can also help guide clinical practice and policy with regards to the frequency of updating patient information. A comparison of 1-year versus 2-year measurements of FEV1%, for instance , showed minor differences in performance.

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Conclusion

In this study we demonstrated that employing a statistical evaluation approach that's closely tied to the clinical goal of using predicted risk as a score to rank patients as a function of time can significantly change the conclusions drawn about a risk prediction model ' s performance. As new models are developed, perhaps incorporating novel biomarkers, the proposed approach might be wont to accurately assess their predictive ability. As shown, standard methods may underestimate their performance by not capturing how these models are going to be used dynamically within the clinical setting. We note that our focus here is on risk prediction models, assuming that patients are added to a lung transplantation wait-list supported their expected enjoy transplantation. In practice, any risk prediction should be including assessments of treatment benefit. It is imperative to still develop models that accurately predict survival in CF. Our proposed approach can function the idea for evaluating the predictive ability of those models by better accounting for his or her dynamic clinical use.

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