Abstracts

PCV73

USE OF PROPENSITY SCORE METHODOLOGY IN CARDIOVASCULAR DEVICE TRIALS: U.S. FOOD AND DRUG ADMINISTRATION PERSPECTIVES

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OBJECTIVE: Randomized, controlled trials (RCTs) are considered to be the gold standard of scientific evidence to assess safety and effectiveness of cardiovascular devices. However, RCT use is challenging to implement in certain device trials, due to logistical and ethical reasons. The FDA understands that assessment of device technologies must balance the competing demands of maximizing scientific validity against the practical realities of performing (and effectively completing) these clinical studies. Hence, non-randomized clinical trials are sometimes used in device evaluation. Propensity score analysis, as an alternative to randomization, has been increasing in popularity as a technique to control for baseline differences between treatment groups in non-randomized cardiovascular device studies. METHODS: Propensity scores provide a convenient methodology for covariate adjustment when multiple covariates are involved. However, propensity score methodology does not eliminate many of the scientific limitations of non-randomized studies compared to RCTs, and should not be viewed as a substitute for performing a randomized study. In using propensity score modeling, a full pre-specification of covariates to be included and the model to be used is recommended to minimize the concern of bias introduced by post hoc model development. RESULTS: Furthermore, sensitivity analysis should be performed to demonstrate the robustness of study outcome in the face of hidden bias due to unmeasured or unquantifiable covariates. Lastly, it is recommended that conventional covariate adjustment as well as propensity score adjustment should be performed to demonstrate consistency of outcomes between techniques. CONCLUSION: Propensity score methodology has increased in popularity for covariate adjustment in non-randomized cardiovascular device studies. However, there are limitations to this methodology, which must be fully appreciated to avoid erroneous inferences from study data. Randomized trials are still preferred and strongly encouraged whenever possible, especially for the evaluation of novel cardiovascular devices.

PCV74

A BUDGET IMPACT MODEL FOR EPLERENONE IN THE TREATMENT OF HEART FAILURE POST MYOCARDIAL INFARCTION

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OBJECTIVES: The Eplerenone Post-Acute Myocardial Infarction Heart Failure Efficacy and Survival Study (EPHESUS) showed that the addition of eplerenone to optimal medical therapy reduced both morbidity and mortality in patients with acute myocardial infarction (AMI) complicated by left ventricular dysfunction and heart failure whilst reducing the number and duration of heart failure re-hospitalisations. A budget impact model was developed to estimate the effects of adding eplerenone to standard care in the UK National Health Service (NHS).

METHODS: Within the model the efficacy of eplerenone is based on the EPHESUS study. This is applied to UK epidemiological data on the incidence of AMI, proportion of survivors developing heart failure and their prognosis. UK drug acquisition costs and NHS hospital inpatient costs and average length of stay for England are included. All costs are expressed in pounds sterling. The model estimates the incremental costs and benefits of adding eplerenone to standard care in heart failure resulting from AMI from the perspective of NHS health care decision makers over a three-year period. Input variables include population, incidence of AMI and annual rate of eplerenone uptake.

RESULTS: If all eligible patients are treated in an NHS Primary Care Trust of population 250,000, the estimated cost per life year saved is £6,701 pounds in year three, for an additional expenditure of £256,959. This level of treatment results in a reduction of 101 bed days for re-hospitalisations due to heart failure, at a cost per bed day avoided of €1,207.

CONCLUSIONS: With hospital inpatient care the biggest single health care cost in heart failure, reduction in hospitalisation is a key priority within the UK NHS. Models such as the one described here enable the economic consequences of using a new drug to be identified and clarify the role of drug treatment in delivering NHS priorities.