Abstract:

The current paradigm for randomized clinical trials provides effective protection against type I errors, but a weak basis for defining an intended use population. Use of the eligibility criteria as a basis for inferring “who benefits” from the test treatment in positive clinical trials often results in over-treatment of patient populations, very small average treatment effects and large NNT (number needed to treat for each patient who benefits). I will describe a new predictive paradigm for “subset analysis”. The focus is on development of an internally validated predictive classifier rather than on inference based multiple post-hoc testing. This Predictive Analysis of Clinical Trials can provide a useful supplement to the primary test of the global null hypothesis for using the results of the trial.