



Nonparametric tests for multistate processes with clustered data

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Abstract

In this work, we propose nonparametric two-sample tests for population-averaged transition and state occupation probabilities for continuous-time and finite state space processes with clustered, right-censored, and/or left-truncated data. We consider settings where the two groups under comparison are independent or dependent, with or without complete cluster structure. The proposed tests do not impose assumptions regarding the structure of the within-cluster dependence and are applicable to settings with informative cluster size and/or non-Markov processes. The asymptotic properties of the tests are rigorously established using empirical process theory. Simulation studies show that the proposed tests work well even with a small number of clusters, and that they can be substantially more powerful compared to the only, to the best of our knowledge, previously proposed nonparametric test for this problem. The tests are illustrated using data from a multicenter randomized controlled trial on metastatic squamous-cell carcinoma of the head and neck.

Keywords Cluster randomized trial · Informative cluster size · Multistate model · Multicenter · Two-sample test

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