NONPARAMETRIC REGRESSION ANALYSIS OF RESTRICTED MEANS AND QUANTILES IN THE PRESENCE OF RIGHT CENSORED DATA

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The hazard ratio is commonly used for comparing survival distributions across groups. While easily estimated in the presence of censored data, it does not allow physicians, patients, and regulatory agencies to judge the clinical relevance of any difference in survival across groups. We consider an approach to nonparametric inference for clinically meaningful functionals of a survivor distribution (e.g., the restricted mean, quantiles). In this approach we use different regression models to borrow information across sparse data than to form statistical contrasts of an estimated functional of interest. We illustrate the general approach in the context of recursive partitioning of a multivariate predictor space to derive groups that are homogeneous with respect to survival distributions. Nonparametric estimates of the survival distribution within leaves of the regression tree are then used to compute estimates of the 75th percentile and the restricted mean survival. Contrasts across estimates are evaluated in linear regression models, with inference derived using bootstrapped standard errors. This approach was superior to the Cox proportional hazards or Buckley-James approaches when semiparametric model assumptions were violated, though there was loss of efficiency when such assumptions do hold. We conclude with a discussion of extensions to this approach which might provide greater efficiency.