

Challenge and promise of observational studies in cancer research

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Abstract: An alternative source of data from randomized controlled trials can be found in the large observational databases and longitudinally-followed patient cohorts that have emerged. These invaluable resources present new opportunities in research to provide potential insights into cancer treatment and patient care. However, such studies are not without their own set of challenges. The complexity of sampling mechanisms and various biases associated with prospective observational studies raise considerable analytical challenges in both the design and the data analysis. The peril of selection bias is exacerbated in many cohort studies. To address the above challenges, we need practical statistical designs and innovative analytic approaches to evaluate clinical effectiveness and healthcare interventions outside of controlled clinical trials. We will give an overview on recent semiparametric modeling for right-censored survival data under length-biased sampling. The methods will be reviewed for commonly used proportional hazards model, and AFT model for time-to-event outcomes, and restricted mean survival times. Some related software for the implementation of such methods will be illustrated.