Take-home exercise on pseudo-values

XVII Summer School of the Master's degree in Statistics and Operations Research

Course: Multi-state models: Rates, risks, and pseudo-values

TO BE SUBMITTED NO LATER THAN 5 JULY 2024
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During the course you have gained experience with analysing pseudo-values (or pseudo-observations) using the packages `pseudo` and `geepack`. The package `eventglm` is able to calculate and analyse pseudo-vales in 'one go', i.e., to calculate the pseudo-values, add to the data set, and then do the regression analysis. Please, familiarize yourself with the `eventglm` package and use it to re-do some of the exercises from day 4 & 5 inserted below (i.e., using the PBC-3 data). Make a brief report of the results including code, output, and some interpretations.

First, consider the composite endpoint *time to death or transplantation.*

1. Estimate the risk difference between the two treatments at year 2, using pseudo-observations (POs) and the 'identity' (`id`) link function.
2. Repeat 1. adjusted for `alb` and `log2(bili)`.
3. Repeat 2. using the `log` link function, i.e., targeting the risk ratio.
4. Repeat 2. using the `cloglog` link function, i.e., targeting the hazard ratio.
5. Fit a joint model based on POs at year 1, 2, 3, and 4 using the `cloglog` link function and `tment` as the only covariate.
6. Repeat 5. adjusted for `alb` and `log2(bili)`.
7. Using the package `eventglm` for several time points and having a covariate with missing values is different than using `pseudo` and subsequent `geepack` (compare the estimate for `tment` to the one from exercise 7 on day 4). Can you think of an explanation for the difference?
8. Estimate the RMST difference at year 3 between the two treatments using POs and the 'identity' (`id`) link function.
9. Repeat 8. adjusted for `alb` and `log2(bili)`.

Now, consider the competing risks situation with the two event types *transplantation* and *death without transplantation* and focus on transplantation only.

1. Estimate the risk difference between the two treatments at year 2, using POs for transplantation and the 'identity' (`id`) link function.
2. Repeat 1. adjusted for `alb` and `log2(bili)`.
3. Repeat 2. using the `cloglog` link function.
4. Fit a joint model based on POs for transplantation at year 1, 2, 3, and 4 using the `cloglog` link function and `tment` as the only covariate.
5. Repeat 4. adjusted for `alb` and `log2(bili)`.
6. Estimate the difference in expected years lost due to transplantation at year 3 between the two treatments using POs and the 'identity' (`id`) link function.
7. Repeat 6. adjusted for `alb` and `log2(bili)`.