



Assessing Treatment Benefit in Immuno-oncology

Adaptations to tumor response and progression criteria for immune therapies.

Abstract

Immuno-oncology is a buoyant field of research, with recently developed drugs showing unprecedented response rates and/or a hope for a meaningful prolongation of the overall survival of some patients. These promising clinical developments have also pointed to the need of adapting statistical methods to best describe and test for treatment effects in randomized clinical trials.

We review adaptations to tumor response and progression criteria for immune therapies. Survival may be the endpoint of choice for clinical trials in some tumor types, and the search for surrogate endpoints is likely to continue to try and reduce the duration and size of clinical trials. In situations for which hazards are likely to be non-proportional, weighted logrank tests may be preferred as they have substantially more power to detect late separation of survival curves. Alternatively, there is currently much interest in accelerated failure time models, and in capturing treatment effect by the difference in restricted mean survival times between randomized groups.

Finally, generalized pairwise comparisons offer much promise in the field of immuno-oncology, both to detect late emerging treatment effects and as a general approach to personalize treatment choices through a benefit/risk approach.

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