RESEARCH ROUNDUP

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Healthcare decision makers (whether they are payers, regulators, clinicians, or health economists) have to grapple with a variety of evidence presented to them. Interpretation of Kaplan-Meir plots or response rates are but 2 presentations of that evidence, and we have selected 2 recent articles that discuss the presentation and interpretation of these data. Finally, qualitative health-preference research also can be utilized, and we present a paper that discusses a set of guidelines to improve the frequency and quality of reporting.

Proposals on Kaplan–Meier plots in medical research and a survey of stakeholder views: KMunicate

Morris T, Jarvis C, Cragg W, Phillips P, Choodari-Oskooei B, Sydes M. *BMJ Open*. 2019;9(e030215): doi:10.1136/bmjopen-2019-030215

Summary

We all use Kaplan-Meier curves or plots, but how is the information best communicated to both decision makers and non-decision makers? What is the level of uncertainty in the difference estimates in survival time between the treatment groups? In this *BMJ Open* article, Morris, et al present research on improvements that can be made to the presentation of Kaplan-Meier curves to show the status of patients over time, and to illustrate the uncertainty of the estimates. The authors then survey stakeholders in order to understand which improvements are preferred. The authors created 6 improvements of the "standard" Kaplan-Meier plot from 3 published phase III randomized trials, and surveyed 1174 participants over a 6-week period. Most proposals were more popular than the "standard" Kaplan-Meier plot. The most popular proposals were in 2 categories:

1. An extended table beneath the plot depicting the numbers at risk, censored and having experienced an event at periodic timepoints.

2. Confidence intervals around each Kaplan-Meier curve, the latter one a favorite of mine.

Relevance

The presentation of an extended table beneath the plot depicting the numbers at risk (Plot A in Figure 2 of the paper), together with confidence intervals around the estimates (Plot E in Figure 2), would greatly increase the ability of both expert and non-expert decision makers to understand the survival times more easily. Kaplan-Meier plots remain an important tool in research and analysis and the development of a more visually meaningful presentation of the result is a great step forward.

Reporting formative qualitative research to support the development of quantitative preference study protocols and corresponding survey instruments: guidelines for authors and reviewers

Hollin I, Craig B, Coast J, Beusterien K, Vass C, DiSantostefano R, Peay H. *Patient*. Published online: December 2019.

Summary

Hollin, et al have developed a set of guidelines for authors and reviewers to improve the frequency and quality of reporting of quantitative health preference research. The guidelines focus on formative qualitative research used to develop robust and acceptable quantitative study protocols and corresponding survey instruments in health preference research.

The guidelines have 5 components with subcomponents:
1. Introductory material (4 domains)
2. Methods (12)
3. Results/findings (2)
4. Discussion (2)
5. Other (2)

Relevance

Qualitative research is not often published, but the publication of formative qualitative research is a necessary step toward strengthening the foundation of any quantitative study. These guidelines should aid researchers, reviewers, and regulatory agencies, and at the same time, promote the transparency within health preference research.

Response rates and durations of response for biomarker-based cancer drugs in nonrandomized versus randomized trials

Gyawali B, D'Andrea E, Franklin J, Kesselheim A. *J Natl Compr Canc Netw*. 2020;18(1):36-43. doi:10.6004/jnccn.2019.7345

In this original research article, Gyawali, et al evaluated whether the response rates and durations of response of targeted cancer drugs observed in nonrandomized controlled trials (non-RCTs) are consistent when these drugs are tested in randomized controlled trials (RCTs). The authors compared the response rates and median durations of response in non-RCTs versus RCTs using the ratio of response rates and the ratio of durations of response (defined as the response rates [or durations of response] in non-RCTs divided by the response rates [or

durations of response] in RCTs). The ratio of response rates or durations of response was pooled across the trial pairs using random-effects meta-analysis. Both non-RCTs and RCTs were available for 19 drug-indication pairs selected. The response rates and durations of response in non-RCTs were greater than those in RCTs in 63% and 87% of cases, respectively. The pooled ratio of response rates was 1.06 (95% CI, 0.95–1.20), and the pooled ratio of durations of response was 1.17 (95% CI, 1.03–

1.33). Response rates and durations of response derived from non-RCTs were also poor surrogates for overall survival derived from RCTs.

Relevance

As more and more drugs, especially new targeted cancer drugs, are slated to receive regulatory approval globally, based on durable responses in non-RCTs, this is important research to consider. A critical eye should be cast over the use of durable responses data derived from non-RCTs, because the responses could be overestimates and poor predictors of survival benefit. The authors conclude that caution must be exercised when approving or prescribing targeted drugs based on data on durable responses derived from non-RCTs.

Note: The preceding texts are simplified summaries of the published articles. They do not contain an opinion on an in-depth analysis the results obtained by the authors. The selection of these works was made based on overall relevance to the HEOR community, not a product of a literature review or of a methodological quality selection.