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Response Adaptive Randomization Based On Activity Digital Outcomes In Cancer Trials

Maria Vittoria Chiaruttini
Unit of Biostatistics, Epidemiology and Public Health, Department of Cardiac, Thoracic, and Vascular Sciences and Public Health, University of Padova, Padova, Italy

Maria Vittoria Chiaruttini
Please provide a brief biography for the Presenting author(s)
Third year Ph.D student in biostatistics and clinical epidemiology at the Unit of Biostatistics, Epidemiology, and Public Health, University of Padova, Italy. Currently, her PhD project focuses on advanced topics in clinical trial design and analysis both in cardiovascular and oncological confirmatory studies.
Her field of interest includes group sequential designs and multiple hypothesis testing, historical information borrowing in new evidence, and response adaptive randomization.

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Non-inferiority trials in oncology aim to demonstrate that an experimental treatment is not clinically inferior to the standard of care. This approach is particularly valuable for treatments that have comparable efficacy but can reduce the side effects of invasive therapies such as chemotherapy, thereby improving patients' quality-of-life. In cancer care, improving QoL is critical because both the disease and its treatments have a significant impact on patients' lives. Therefore, in addition to prolonging life, it is necessary to minimize the side effects of treatment, especially for patients who are no longer curable.

The trial proposes a design using response-adaptive randomization to preferentially assign patients to treatments with evidence of superiority on QoL outcomes. Given the non-inferiority of the primary endpoint, the trial will utilize digital secondary endpoints focused on activity metrics collected by wearable devices. These devices provide real-time, minimally intrusive health indicators, such as daily step counts, to provide a continuous assessment of patients' daily activity. The statistical framework includes Bayesian modelling of digital endpoints and strategies to address data challenges such as seasonality and other sources of variability in activity data.

This approach aims to improve the patient-centeredness of non-inferiority trials adjusting allocation ratios based on real-time activity data. However, several aspects are still under investigation, including the relationship between QoL, drug tolerability, and efficacy outcomes, the maximum imbalance ratio to protect power, and the timing of adaptation. Nevertheless, activity data underscore their potential as practical and informative metrics that captures patient functioning, treatment tolerability, and well-being.