University of California, Irvine
Statistics Seminar

Sample Size Considerations for Precision Medicine

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Join via Zoom: https://uci.zoom.us/j/91370133600

Sequential Multiple Assignment Randomized Trials (SMARTs) are considered the gold standard for estimation and evaluation of treatment regimes. SMARTs are typically sized to ensure sufficient power for a simple comparison, e.g., the comparison of two fixed treatment sequences. Estimation of an optimal treatment regime is conducted as part of a secondary and hypothesis-generating analysis with formal evaluation of the estimated optimal regime deferred to a follow-up trial. However, running a follow-up trial to evaluate an estimated optimal treatment regime is costly and time-consuming; furthermore, the estimated optimal regime that is to be evaluated in such a follow-up trial may be far from optimal if the original trial was underpowered for estimation of an optimal regime. We derive sample size procedures for a SMART that ensure: (i) sufficient power for comparing the optimal treatment regime with standard of care; and (ii) the estimated optimal regime is within a given tolerance of the true optimal regime with high-probability. We establish asymptotic validity of the proposed procedures and demonstrate their finite sample performance in a series of simulation experiments.