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Title: A comparison of approaches to incorporate patient-selected and patient-ranked outcomes in clinical trials

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Abstract

A key aspect of patient centered drug development is identifying and measuring outcomes that are important to patients in clinical trials. Many medical conditions affect multiple symptom domains, and a consensus approach to determine the relative importance of the associated multiple outcomes ignores the heterogeneity in individual patient preferences. Patient-selected outcomes offer one way to incorporate individual patient preferences, as proposed in recent regulatory guidance for the treatment for migraine, where each patient selects their most bothersome migraine-associated symptom in addition to pain. Patient-ranked outcomes have also recently been proposed, which go further and consider the full ranking of the relative importance of all the outcomes. This can be assessed using a composite DOOR (Desirability of Outcome Ranking) endpoint. In this talk, we compare the advantages and disadvantages of using patient-selected versus patient-ranked outcomes in the context of a two-arm randomised controlled trial for multiple sclerosis. We compare the power and type I error rate by simulation, and discuss several other important considerations when using the two approaches.