Widening the spectrum of health outcomes used in health technology assessment: integrated synthesis and mapping to QALYs

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Title of project or programme

Widening the spectrum of health outcomes used in health technology assessment: integrated synthesis and mapping to QALYs

Principal Investigators of project/programme grant

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• United Kingdom

Source of funding information

Medical Research Council

Total sum awarded (Euro)

277512.07

Start date of award

01-01-2010

Total duration of award in months

24

The project/programme is most relevant to

• Alzheimer's disease and other dementias

Keywords Research abstract in English Cost-effectiveness analyses used in NICE appraisals and Clinical Guidelines require that treatment differences on outcomes measured in clinical trials are mapped into a common utility scale. The mapping is based on a regression calibration informed by data from a cohort study or trial. This form of analysis is a particularly common feature in neurology, psychiatry, dermatology, and rheumatology, where treatments are evaluated using physician- or patient-reported assessments.

Current methods are based on mapping from a single outcome, usually the one most often reported. This makes inefficient use of data, focuses attention on inappropriate outcomes, and may result in some trials being excluded. Failure to take account of measurement error, a common feature of these outcome scales, leads to under-estimation of QALY gains due to treatment. More seriously, use of a univariate mapping relation may systematically under-estimate the QALY gains due to treatment, and produce biased estimates of relative treatment differences, especially in diseases like Alzheimer's where treatments may result in imperfectly correlated changes on distinct dimensions of life quality, that cannot be reflected on any single measure.

We propose an integrated approach that synthesises the multiple outcomes reported in trials and simultaneously effects a multi-variate regression mapping onto a QALY scale. It will be possible to combine outcome information even when different trials report different combinations of outcomes. Our approach will also use methods able to combine different regressions in order to synthesise all the information that could inform the multi-variate mapping. We will use published material on test-retest, inter- and intra-rater reliability to provide additional information on measurement error and correlation parameters.

This is a novel treatment of multi-outcome synthesis that takes better advantage of multiple, correlated outcomes than current approaches, by employing the between-measure relationships that are already assumed in mapping. Our method will also allow multiple sources of data to be used to directly inform the mapping, which will enable a systematic use of available evidence, rather than the somewhat arbitrary selection of a single cohort study that characterises current practice.

We propose to apply these methods to Alzheimer's disease, depression, psoriasis and psoriatic arthritis. In each case we will contrast the treatment effects mapped onto the QALY scale produced by the proposed multi-outcome approach to those produced by univariate synthesis. We will also show how the method can be adapted to the Markov state structures commonly used in cost-effectiveness models.

Lay Summary