General appendix

(all papers refer to this appendix)

This appendix describes WHO-CHOICE methods used for estimating the denominator of a cost-effectiveness ratio, i.e. effectiveness. 'Effectiveness' in this context is defined as the difference in health obtained as a result of exposure to an intervention.

WHO-CHOICE uses an aggregate estimate of effectiveness, one which sums up individual changes in health across a population. Like 'burden of disease', the estimate combines mortality rates with information on the severity and duration of time spent in conditions that reduce health (i.e. 'morbidity').

The goal of WHO-CHOICE is to compare interventions in terms of cost effectiveness, which is to say in terms of their economic efficiency. (N.B. Cost estimation is not discussed in this appendix.) Explicit, quantitative information on efficiency encourages decision makers in the health sector to take into account trade-offs that may arise in the course of priority setting, for example, between equity on the one hand and efficiency on the other.

The unit of effectiveness used here is the disability-adjusted life year (DALY). DALYs belong to a family of similar measures, including Healthy Year Equivalents and Quality Adjusted Life Years. DALYs are closely related to standard life-table measures (e.g. Life Years) as well as, more distantly, to the whole class of summary measures of population health (including, for example, Life Expectancy).

The loss of health attributable to non-fatal outcomes first requires an estimate of the condition's incidence. For each case, its duration (i.e. time from incidence to remission or death) is multiplied by a number assigning a severity value.

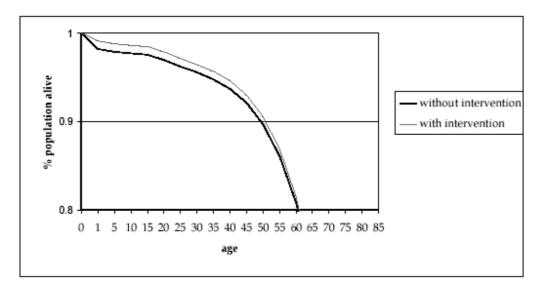
The "severity" of time spent with a non-fatal condition is intended to quantify average societal perceptions regarding how a particular condition compares with full health. Depending on how they are derived, severity weights have been called "disability weights", "health state valuations", "health state preferences" or "health utilities".

Non-fatal outcomes differ in their impact according to individual factors; impact is also mediated by contextual factors including the physical and social environment. Such outcomes can affect multiple domains of functioning including mobility, affect and pain. Thus, a severity score of 0.50 is not intended to imply that an individual with the condition is "half dead", that they experience their life as half as rich as that of a healthy person, or that society values them half as much.

Gains to health (decreases in DALYs) projected to occur beyond the current period are discounted at 3%. The rationale for discounting health benefits is discussed in Murray and Lopez (1996).

DALYs also use age weights, meaning that a year of healthy life lived at very young or old ages counts less than a year lived at intermediate ages. The weights follow a functional form that has been found to match societal preferences for health at different ages. A more detailed discussion can also be found in Murray and Lopez (1996).

A useful way to think about the effect of an intervention is in terms of shifts in the survivorship curve. The survivorship curve is a visual representation of the mortality history of a closed population. In the figure below, the *y*-axis shows the proportion of a birth cohort surviving to a given age, which is shown on the *x*-axis. An intervention reducing infant mortality by 50% would shift the curve upwards as depicted. Years of life lived (i.e. the area under the curve) are increased.



Similarly, population projection models calculate the benefits of an intervention as the difference in the healthy life years lived by a population with and without the intervention (i.e. analogous to the area between the two curves in the figure). WHO-CHOICE developed and uses a population model known as PopMod.

A population model describes the health of a population that is subject to a set of health states (e.g. healthy, ill) and is exposed to the risk of events (i.e. transitions between states). A starting population is defined, and the population's health experience is simulated by 'turning on' model time and allowing events to occur according to observed epidemiological and demographic rates. The simulation is projected forward for 100 years, to allow for the full lifetimes of the starting birth cohort to be captured.

PopMod allows for four different health states, notionally thought of as: full health; index disease X; index disease C; and the joint (co-morbid) condition X + C. Transitions are possible from health to illness (X or C), from illness to health, from a single illness to co-morbidity (and the reverse). Mortality (both disease-specific and other) is accounted for, as are births. More information is provided in Lauer et al. (2003).

At the outset of the simulation, the population of interest is distributed into the defined disease states based on observed or estimated prevalence rates.

Health state valuations are applied to time spent in disease states (e.g. X or C).

To compare population health with and without the intervention, a number of steps are required:

- 1. define the cluster of related interventions.
- 2. define the current epidemiological and demographic parameters, including current disease rates, prevalence and severity.
- 3. estimate the epidemiological and demographic parameters of the null scenario.
- 4. define the population of interest and the starting position of the simulation.
- 5. using the population projection model, estimate the population health under the intervention and null scenarios.
- 6. take the difference between each intervention and null scenario.

A key step here is estimation of the null scenario, which is defined as the level of population health obtained when a defined group of interventions is discontinued. Estimating the null scenario depends on the nature of the interventions involved:

- The elimination of preventive interventions increases the rates of incidence of disease.
- The elimination of curative interventions decreases the rates of remission, or increases the rates of fatality, of disease.
- The elimination of rehabilitative or palliative interventions affects the severity or duration of disease.

In most cases, estimation of the null scenario requires implementing changes in all these parameters.

Interventions affect these same parameters. In WHO-CHOICE, interventions are evaluated assuming they are implemented for a duration of 10 years. The population model is used to translate estimates of intervention efficacy (typically, risk differences or relative risks for outcomes) into estimates of intervention effectiveness.

Data sources on intervention efficacy are described in each of the papers.

References

Lauer JA, Rohrich K, Wirth H, Charette C, Gribble S, Murray CJL. PopMod: a longitudinal population model with two interacting disease states. *Cost-effectiveness and Resource Allocation 2003*; 1(6). Available at: <u>http://www.resource-allocation.com/content/1/1/6</u>.

Murray CJL, Lopez AD. The Global Burden of Disease: a comprehensiveassessment of mortality and disability from diseases, injuries, and risk factors in 1990 and projected to 2020. 1st ed. Murray CJL, Lopez AD, eds. Cambridge, MA: Harvard University Press; 1996.