The appraisal of public health interventions: an overview

A.J. Fischer1,2, A. Threlfall3, S. Meah3, R. Cookson4, H. Rutter5, M.P . Kelly1,2

1Centre for Public Health Excellence, National Institute for Health and Care Excellence (NICE), London, UK
2Health Sciences, University of Manchester, UK
3Formerly Greater Manchester Public Health Practice Unit, Manchester, UK
4Centre for Health Economics, University of York, York, UK
5London School of Hygiene and Tropical Medicine, London, UK

Address correspondence to A.J. Fischer, E-mail: alastair.fischer@nice.org.uk

ABSTRACT

Background The approach currently used to appraise public health interventions is close to that of health technology appraisal for drugs. This approach is not appropriate for many public health interventions, however, when extremely small individual level benefits are delivered to extremely large populations. In many such situations, randomized controlled trials with sufficient size and power to determine individual level effects are impractical. Such interventions may be cost-effective, even in the absence of traditional evidence to demonstrate this.

Methods We outline an alternative approach based on decision theory. We apply it to cases where prior beliefs are sufficiently strong and well grounded to allow decision-makers to assume the direction of change of the intervention’s outcome, within the context of a transparent and deliberative decision-making process. Decision theory also assumes that decision-makers are risk neutral, implying that they should make decisions based on an intervention’s mean cost-effectiveness, and should therefore disregard variance except when deciding to wait for more information. However, they must allow for biases.

Results A framework is presented which has the potential to achieve large health gains at no additional cost.

Conclusions This analysis provides a rigorous theoretical framework for decision-makers in public health. The implied paradigm shift also applies to some clinically based areas.

Keywords economics, methods, public health, decision theory

Introduction

Significant progress has been made in the last 25 years in ensuring that decisions about people’s health and the investment of public money are underpinned by the best possible scientific evidence. Organizations such as the UK’s National Institute for Health and Care Excellence (NICE) and bodies such as the Cochrane Collaboration gather international evidence to support decisions about which interventions can be demonstrated to be effective and worthy of public spending. The appraisal methods used by NICE to guide decisions about health technologies are, with few modifications, applied to the appraisal of public health interventions. These methods of appraisal are based on the principles and practice of evidence-based health care1–3 [The methods used in Health Technology Assessment (HTA) are well suited to clinical interventions]. However, the fit of these methods for the assessment of public health interventions is less than exact.4 This paper suggests that the application of appraisal methods designed for clinical scenarios inhibits spending on community-based public health interventions. It argues for using decision theoretic approaches in appraising public health interventions to underpin judgements about investment.

Currently, NICE’s systems of appraisal are designed to demonstrate that interventions about which interventions can be demonstrated to be effective and worthy of public spending. The appraisal methods used by NICE to guide decisions about health technologies are, with few modifications, applied to the appraisal of public health interventions. These methods of appraisal are based on the principles and practice of evidence-based health care1–3 [The methods used in Health Technology Assessment (HTA) are well suited to clinical interventions]. However, the fit of these methods for the assessment of public health interventions is less than exact.4 This paper suggests that the application of appraisal methods designed for clinical scenarios inhibits spending on community-based public health interventions. It argues for using decision theoretic approaches in appraising public health interventions to underpin judgements about investment.

Currently, NICE’s systems of appraisal are designed to demonstrate that interventions are clinically effective and safe before progressing to assess cost-effectiveness. Underlying this approach is the assumption that intervention outcomes are well understood and that randomization is the best way to measure them. However, this may not be the case for all public health interventions. Many interventions have such small individual benefits that they are not feasible to study in randomized controlled trials. In such situations, it may be more effective to use decision theory to make decisions about whether to proceed with the intervention. This analysis provides a rigorous theoretical framework for decision-makers in public health. The implied paradigm shift also applies to some clinically based areas.
processes include comprehensive searches of the world’s knowledge base, scrutiny of methodological quality and appropriate use of meta-analysis to allow conclusions to be drawn. Appropriate steps are taken to ensure that causal inferences are valid and to address possible biases and confounding factors. Conclusions about effectiveness and safety can be traced back to a set of published empirical studies identified through a predefined search strategy. At NICE, safe and effective interventions are assessed against a threshold of cost-effectiveness; depending on the outcome, a recommendation is made as to whether the intervention offers sufficient benefit for public funding.

The HTA methods used by NICE have become an accepted standard, supporting people responsible for making decisions on behalf of the public as to whether interventions and services should be funded and available. Randomized control trials (RCTs) are used where possible, because they are designed both to minimize bias and to be of sufficient size to ensure that improvements in healthcare are real and not due to chance. The investigators’ prior beliefs (e.g. beliefs about the effectiveness of the intervention) held before the RCT or observational evidence has been obtained are not taken into account in this process. This approach is illustrated by published HTAs from (for example) the NIHR Health Technology Assessment programme. A problem arises for decision-makers when there are either no RCTs, or RCTs which are not of sufficient size to determine effectiveness with sufficient certainty. This is often the case when considering community-based public health interventions. In such cases, of course, decisions continue to be made and are highly influenced by prior beliefs and experiential knowledge. To resolve this dilemma and create a scientifically robust, systematic and transparent approach to support decision-makers, we argue that a decision-theory approach should be applied. Indeed, methods used by NICE to establish the cost-effectiveness of public health interventions already take this approach: this paper provides the theoretical underpinnings for this approach and argues for its more widespread and systematic application.

A decision-theory approach can be applied to all health evaluations, not just the appraisal of public health interventions. The benefit of using a decision-theoretic approach is that it enables decision-makers to use their prior beliefs, in a formal Bayesian structure, to inform the decision-making process.

Decision theory, we suggest, should be applied to all forms of community level public health interventions with small individual health benefits that potentially add up to large population health benefits. This extends to programmes such as screening, immunization and secondary prevention; to behaviour-change driven by up-stream interventions such as taxes, subsidies, regulations and ‘nudges’ towards healthy behaviour delivered either inside or outside the health sector and to broader social and economic policies with important health impacts, including the environment, transport, occupational safety, education, housing, tax and social security. The rationale for this approach is that community-level interventions of this type often have large population-level health effects, but small individual-level health effects that are difficult if not impossible to detect in RCTs without extremely large sample sizes of tens of thousands of individuals, or more. In practice, RCT sample sizes above a thousand in public health are usually only ever seen in studies appraising programmes such as breast screening and are most unusual for the appraisal of smaller scale interventions. Consequently, for many public health interventions, the study evidence base is weak, and outcomes have high levels of uncertainty. Increased investment in well-designed studies at a sufficient scale to address this issue is unlikely, especially given the current economic austerity. Fortunately, a decision-theoretic methodology is available to help decision-makers reach the best possible decisions and avoid the option of rejecting an intervention that is likely to be beneficial on the basis of an absence of adequate evidence. The alternative is reversion to opaque opinion-based decision-making. The practical application of decision theory to decision-making in this context offers a balanced analytical assessment of whether an intervention is likely to be worthwhile, by making intelligent use of all the best available evidence, including theoretical evidence and empirical evidence from observation and natural experiments as well as RCTs.

The application of a decision-theoretic approach to evaluate public health interventions

Our aim is to apply a decision-theoretic approach when evaluating public health interventions to enable decisions to be made in favour of interventions that increase population health at an acceptable cost. This would, for example, happen if public health interventions that are cost-effective and highly unlikely to damage health, e.g. limiting traffic speeds to 20 mph in many residential areas, were implemented. There are of course further non-health considerations that decision-makers would need to consider and weigh in the balance, such as the freedom of choice and convenience of motorists, but our focus in this paper is on the appropriate estimation of health impacts. At an aggregate level, public health interventions are required not to cause harm to health. Harm to health can occur from intervening, or from doing nothing.
The decision to act (or not) should therefore be based on all relevant knowledge about the health impacts of intervening compared with not intervening. The proposed approach appraises interventions within a non-hierarchical framework that allows a weight to be given to all the relevant knowledge and evidence. This is centred on the basic Bayesian notion that probability estimation is a matter of appropriately revising and updating existing prior beliefs in accordance with the available evidence in order to generate posterior beliefs, and thus reach a rational and informed understanding for use by decision-makers.9 Posterior beliefs are those that take account of both prior beliefs and the information from RCTs and observations. Decision theory employs a Bayesian framework which uses the optimal combination of prior information and current information from trials or observations to form posterior beliefs. See Box 1.

Public health interventions aim to deliver an overall population health gain. It is accepted that some individuals will not benefit, or may experience a degree of harm. For example, the introduction of car seat belts resulted in significant gains in preventing injury and death from hitting the windscreen, but some individuals experienced chest injuries from the pressure of the seat belt on impact. Viewed from before a collision occurs, individuals are on average better off wearing seatbelts than not doing so. After a collision, some people will be worse off wearing a seat belt than they would have been had they not worn one. If we could determine who those people would be in advance, we could ensure that no-one was made worse off by wearing a seatbelt. But the decision to wear seatbelts has to be made before the collision occurs, and is made on the basis of whether the intervention on average does more good than harm, and on whether those being harmed can be recognized in advance.

A core tenet of decision theory is that the decision-maker is risk neutral, that is, takes no account of risk. It is most unusual for individuals in their daily lives to be risk neutral. In most circumstances, individuals are risk averse and only usually become risk lovers as gamblers. However, decision-makers in large organizations, who make many decisions, may pool the risk of their decisions over many projects, and that is the sense in which they may ignore the risk.10–12 This also enables the pooling of harm and health gain within a project, as is shown in the seatbelt example above. This allows the recommendation of public health interventions which produce net benefits at a population level, even though it is realized that some individuals within the population, whose identities are unknown in advance, will be harmed.

The initial step in the proposed process is to search and review all relevant available knowledge and evidence to enable evaluators or decision-makers to consider the direction of

---

**Box 1 Informative prior beliefs**

In Bayesian decision theory, the best estimate of the health effect of an intervention need not depend exclusively on evidence from RCTs but can also depend on prior beliefs based on theory, observation and experience. The basic idea is that one starts with an initial set of beliefs and then updates those beliefs as new evidence becomes available. The initial beliefs are called the ‘prior distribution’ and the updated beliefs are called the ‘posterior distribution’. In some cases it may be appropriate to use a ‘null’ or ‘uninformative’ prior and rely exclusively on RCT evidence. In other cases, however, one can use ‘informative prior beliefs’—as illustrated below.
change that an intervention will cause and the likelihood that it will produce a net benefit to health in the population of interest. This is consistent with the evidence-based model, but in this context, evidence under consideration would not be limited only to RCT evidence. It should be noted when considering the benefit and harm from a public health behavioural intervention that harm is often highly unlikely because the intervention often involves an attempt to encourage one part of the population to behave like another, healthier, part. For example, people who do not eat fruit and vegetables regularly might be encouraged to follow those eating five portions or more fruit and vegetables a day. In this context, observation of populations may provide a high level of understanding about net outcomes and thus a high level of confidence about the direction of change the intervention would cause. For example, knowledge derived from observation, experience, published literature and some small studies supports the provision of free fruit in the workplace as a mechanism likely to result in improved nutrition, with minimal risk of harm to health for any individual. The absence of evidence from RCTs to support interventions of this kind should not prohibit a decision to use the intervention. Ironically, when very small and underpowered studies are available, a reliance on these sources of evidence alone is more likely to lead to a lack of action than if knowledge from an appropriate theory is the only source of information about an intervention. Following consideration of all relevant available knowledge and evidence leading to the belief that the intervention may produce some benefit, cost-effectiveness must still be considered, because the intervention might be too costly to implement for the level of benefit likely to accrue.

To establish the cost-effectiveness of an intervention, the ratio of the incremental cost per unit of benefit (such as the quality-adjusted life year (QALY) gain) is estimated and is compared with its opportunity cost (i.e. the cost per QALY of the next best alternative use of the funds, which in the UK is believed to be £20,000 per QALY). If all we know about the benefits is that they are positive but we do not know their magnitude, and we have an estimate of incremental costs, we can establish the minimum size of the benefits that would allow the cost-effectiveness ratio to be <£20,000 per QALY. If it is believed that the actual benefit will exceed this threshold level of benefit, then the intervention should be approved. In a decision-theoretic approach, the requirement that the decision-maker is risk neutral means that the variance in an estimate of either cost or effect is not considered. Hence, when available from studies, the estimated mean effectiveness can be used without regard to that part of uncertainty due to chance, but bias of a known direction cannot be ignored. However, pooling still applies to what has been called ‘second-order uncertainty’ such as not knowing the rate at which people wish to discount the future.11

The concept of ‘transparency’ is fundamental to the decision-theoretic approach. Being wholly transparent about prior beliefs regarding a public health intervention—and the process, knowledge and evidence used to develop subsequent posterior beliefs that underpin recommendations—is vital to enable open consultation and challenge as part of the decision-making process. Transparency also offers a mechanism to protect the proposed approach from the effect of bias, particularly in the form of publication bias (studies with negative or inconclusive results being less likely to be published) and bias from self-interest and prior prejudice among decision-makers in the form of ‘pet theories’.13–15 Transparency also allows open consideration of the timing of a decision including whether it is better to wait for, or recommend the collection of, more evidence before a decision is made.

Using evidence and theory to support decision-making

The availability of research funding to conduct large RCTs is limited. The sample size required to ensure statistical significance can be predetermined when investigating whether or not an intervention will be successful. The effect size at an individual level of many public health interventions is very small, and to try to ensure that an inconclusive result is avoided would require an extremely large sample size. The cost of such a large trial will usually prohibit researchers from undertaking it. A consequence of this is that many existing systematic reviews and meta-analyses fail to determine whether public health interventions are effective. The question arises as to how decisions can be made about the application of interventions to address public health problems in the absence of RCT evidence, where that RCT evidence is inconclusive. The decision-theoretic approach offers an opportunity to expand the type of knowledge examined within the evidence base for decision-making, to include knowledge that is in the public domain but is not derived from RCTs or from estimation of statistical significance. Thus, consideration is given to theoretical and empirical knowledge derived from natural experiments, observation and experience. Consideration of this wider evidence base, alongside any RCT evidence that is available, leads to a set of posterior beliefs about the effectiveness of the intervention to offer population health benefit. There are many possible examples where such an approach is the norm, e.g. to alleviate the impact of natural disasters, there are no RCTs to tell us the best ways to intervene. (How could there be?) Nevertheless, it is possible to be
confident that a proposed disaster response intervention, compared with doing nothing, will not have an effect in the wrong direction. In this case, our prior knowledge alone (from evidence other than from trials) is sufficient to allow us to act appropriately. It is therefore apposite to go straight to a consideration of the cost-effectiveness of such interventions, by making a judgement about the minimum size of the effect (for a given level of spending) that is necessary for cost-effectiveness. The proposed approach offers a unified decision analytical framework which allows the integration of ‘comparative effectiveness review’ and ‘cost-effectiveness analysis’. Analysts are thus enabled to use prior knowledge (about the direction and size of health effects) that is sufficiently well informed not to require further RCT or additional

**Box 2 Salt and hypertension**

Moderate reductions in the amount of salt people eat doesn’t reduce their likelihood of dying or experiencing cardiovascular disease. This is the main conclusion from a systematic review published in the latest edition of the Cochrane Library. Cochrane press release, 5 July 2011 from ref. 16.

Moderate reductions in the amount of salt people eat will have a relatively small effect over a period of a few months, so to show a statistically significant reduction in deaths or in the incidence of cardiac disease would require very large sample numbers. When all the studies that were analysed in Taylor et al.16 were amalgamated into a meta-analysis, the combined sample size was not large enough to conclude that there was a significant effect. However, the press release that says that moderate increases in the amount of salt people eat ‘doesn’t reduce their likelihood of dying or experiencing cardiovascular disease’ is a short-hand for ‘doesn’t significantly reduce their likelihood of dying or experiencing cardiovascular disease’. It is quite false to suggest that the true effect was zero: rather, we ‘know’ from a range of other studies and biological plausibility that the true effect is likely to be small, but that the chance that it is exactly zero is remote. Additionally, we ‘know’ from other sources that a reduction in salt eaten is extremely unlikely to cause higher mortality. Importantly, cost-effectiveness of interventions that might achieve reductions in salt intake is not ruled out by the findings of the Cochrane study.16 The estimated relative risk for a number of different outcomes embedded within Taylor et al.’s16 meta-analyses in every case considered is always in the expected direction (but not significant). Others17 believe that when one trial that they think is inappropriately included in the analysis is excluded, the interventions to reduce salt intake would become effective at the 95% level of confidence. While that may be the case, these authors are still trying to justify a policy to reduce salt consumption using the standard HTA or so-called ‘frequentist’ approach. According to a decision-theoretic approach, the main reason for believing that Taylor’s16 analysis is too conservative is that theory and observational evidence are not appropriately considered. Putting complete weight behind a set of trials that we know to be underpowered and saying that we ignore all other theory and forms of evidence may be in accord with an HTA framework, but does not accord with a decision-theoretic framework. If, in the process of using the proposed approach, it were accepted that there are flaws in past theories and observations and it is unsafe to rule out the possibility that reducing salt intake actually increases mortality and cardiovascular events, then we should heed the results of the RCTs and be cautious. Otherwise, we should go straight to cost-effectiveness.

For this example, the cost-effectiveness evidence is overwhelming. Legislation or working with the food industry on reformulation to reduce the amount of added salt in food would be cheap to implement and police after the initial transition costs. It is estimated18 that the cost per DALY gained from such legislation in Australia would be AU$630, or of the order of £400 (UK) per QALY gained, assuming that DALYs and QALYs are interchangeable in this context. If the NHS in England were prepared to pay £20 000 per QALY gained, and a gain of 260 000 QALYs were to eventuate (scaling up the estimated 110 000 DALY gain in Australia by relative population sizes) then the amount the NHS would have to put aside to get the same health gain by other means would be up to £5.1 billion (= £(20 000–400) times 260 000). This shows the order of magnitude of the possible gain. It assumes that the 260 000 QALYs are gained immediately rather than over time and takes no account of future costs or cost savings, so must be regarded as indicative of large gains rather than as a definitive £5 billion gain. If biases such as publication bias are considered, the estimated net gains will be smaller again, but it is reasonable to suppose that they would still far outweigh the cost of legislation.

The examples show that potentially cost-effective interventions that should result in very large health gains (that the NHS would have to pay dearly for, in order to get the same gains by other means) may well be missed by not taking accepted and reliable theoretical evidence into account. The money thus ‘saved’ could easily add up to some billions of pounds. In this case, not using a formal decision theoretic model, but rather inferential inductive reasoning, the Programme Development Group which developed the NICE guidance on the prevention of cardiovascular disease did conclude that a population approach to reducing salt intakes was justified and made that recommendation. Informally, they reached the same conclusion as a formal decision-theoretic one, a decision that they might well not have reached by applying standard evidence-based medicine in public health.
empirical evidence. Such prior knowledge is called an ‘informative prior’. It draws on diverse sources of observation, theory and opinion (experts, decision-makers and stakeholders) elicited through a deliberative decision-making process.

For a second example, *Salt and hypertension*, which appears in Box 2, we extend the evidence base from the source set of the previous sentence (the prior beliefs) by adding information from underpowered RCTs. The combination of these two sources of evidence leads to a set of posterior beliefs. Note that the standard evidence-based medicine approach uses only the RCT evidence, whereas a decision-theoretic approach uses all the evidence (that is the prior evidence in combination with the RCT evidence).

Because some interventions that would not have been recommended using a hypothesis-testing approach would now be recommended using the decision-theoretic approach outlined, better outcomes will be achieved for the same budget than previously. In a further paper, we shall outline where we believe that substantial health gains (without increasing the health budget further) could be made by the application of the methodology applied in this paper. The advocated decision-theoretic approach is not at odds with *realistic evaluation* or other approaches to evaluating complex interventions with multiple interacting components. In formulating prior beliefs about the effectiveness of a complex intervention, one needs theoretical and empirical knowledge not just about ‘what works?’ but about ‘what works for whom in what circumstances in what respects, how and why?’ Complexity, with respect to both the intervention and the knowledge base necessary to evaluate the intervention, does not obviate the need for transparency and analytical rigour in describing and justifying prior beliefs about predicted intervention effects on population health. Decision theory has also been developed further than the seminal work of Savage for example, see Anand. Such developments are beyond the scope of this paper. This paper provides an accepted framework into which most or all of the extensions can fit.

**Conclusion**

Open and transparent decision-making, supported by scientific endeavour, is the foundation of good public health practice. The National Health Service (NHS) has made significant progress in developing evidence-based decision-making regarding HTA and cost-effectiveness of publicly funded interventions and services. The learning from evidence-based medicine as applied by NICE has gone some way to support decision-making about public health interventions. Existing approaches have also highlighted the difficulty in applying clinical models of decision-making to societal public health problems. The use of a decision-theoretic approach to the appraisal of public health interventions offers an alternative, and arguably better, mechanism to support public health decision-makers. The approach is consistent with the original principles advocated by Archie Cochrane, the originator of evidence-based medicine and offers new opportunities for public health decision-makers in local government and the NHS.

**Main finding of this study**

A decision-theoretic framework should be used by decision-makers in public health, in many cases displacing the current framework used in evidence-based medicine.

**What is already known on this topic**

Decision theory has been used as a modelling tool in economic evaluations of health care, but has not been widely recognized as the theoretical framework of choice more generally within public health.

**What this study adds**

Accepted theory may be used as an additional form of evidence. The assumption of risk-neutrality of decision-makers has not previously been emphasized for the allocation of resources in public health. Transparency and deliberation are necessary in the context of this form of decision-making, for accountability, replicability and decreasing biases. The study has the potential for choosing interventions that could substantially increase population health benefits at no additional cost to the NHS compared with current decision-making.

**Limitations of this study**

As in all forms of decision-making, biases are difficult to deal with. The study does not consider very complex decision-making.

**Acknowledgements**

The authors are indebted to Iain Chalmers for his thoughtful and comprehensive comments on an earlier draft of this and two further papers on this topic and to Mark Strong and Hazel Squires for help and suggestions.

**References**


