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SUPPORT Tools for evidence-informed health Policymaking (STP)

16. Going from the evidence to a decision

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Abstract

Background: This article is number 16 in a series of 21 articles on tools for evidence-informed health policymaking. Decisions about health programmes and policies cannot be made based on evidence alone. Judgements are always required, including judgements about whether the anticipated benefits outweigh the downsides, how important the impacts are, the resources that are required to implement the policy or programme, and the extent to which the policy or programme is a priority in relationship to other ways in which those resources might be used.

Objectives: We suggest five questions that can help to clarify the implications and limits of research evidence to inform a health policy decision.

Key messages:

- The following questions can be used to clarify the judgements that are made when going from evidence to a decision:
    1. What is the balance between the desirable and undesirable impacts of the policy or programme?
    2. How confident are we about the likely impacts?
    3. How confident are we about the importance of the impacts?
    4. What are the resource implications of implementing the policy or programme?
    5. Is implementing the policy or programme a priority?
- The most important consideration that drives a decision, or should drive a decision, about a health policy or programme is whether it does more good than harm
- When the net benefit (that is the difference between the desirable and undesirable consequences), is large we are more confident about a decision
- Our confidence in the estimated net benefit depends on judgements of the risk of bias (systematic errors), precision (random errors), the consistency of estimates across studies, and how directly relevant the evidence is
- The net benefit of a policy or programme depends not only on estimates of the desirable and undesirable consequences, but also on how important the different consequences are. Different people may, quite legitimately, have different views about the relative importance of different consequences
- The resource implications of a policy or programme should be considered along with other impacts as a consequence of the policy or programme. The greater the cost, the more likely we are to want larger net benefits (excluding costs), impacts on important outcomes, and compelling evidence
- Decisions about priorities should rest on shared criteria or reasoning, be publicly accessible, be possible to appeal in light of considerations that stakeholders may raise, and should be enforced (to ensure that the first three conditions are met)

Background

This article is number 16 in a series of 21 articles on tools for evidence-informed health policymaking [1]. It is also the first of four articles in this series on making decisions and involving stakeholders in decisions. In this article we will suggest five questions that can help to clarify the implications and limits of research evidence to inform a health policy decision.

Research does not make decisions [2]. Judgements are always required, including judgements about what evidence to use, how to interpret that evidence and our confidence in the evidence [3]. More importantly, decisions about health programmes or policies require judgements about whether the anticipated benefits outweigh the downsides [4]. In addition to judgements about how big the impacts are likely to be, this requires judgements about how important they are, the resources that are required to implement the policy or programme, and the extent to which the policy or programme is a priority in relationship to other ways in which those resources might be used.

If a policy or programme was expected to have large benefits with few downsides and little cost; we were confident about the evidence and the importance of the benefits; and the policy or programme was a clear priority, it would be simple to make a decision. Unfortunately, this is rarely the case. More often complex and difficult judgements must be made under uncertainty.

It is possible to develop rules to guide these judgements. For example, governments may use an implicit or explicit cost-effectiveness threshold to guide decisions about new technologies [5], they may have 'negative lists' and only exclude health technologies for specified reasons, they may have 'positive lists' and only fund technologies that meet specified criteria [6], or they may have a set of principles for deciding on priorities [7]. None of these approaches can be applied easily to decisions about health programmes and policies, which are typically complex and for which there is often uncertainty about their impacts. Moreover, even when a set of rules or principles is used, judgements are still needed, whether these are made implicitly or explicitly.

The questions we propose here do not reduce the need for judgements, but more systematic consideration and discussion of these questions could help to ensure that important considerations are not overlooked and that judgements are well-informed. It could also help to resolve or at least clarify reasons for disagreements, and if these judgements are made transparently, it could help others to understand the reasoning behind health policy decisions. The first four questions are similar to factors suggested by the GRADE Working Group to determine the strength of a recommendation [4].

Questions to consider

The following questions can be used to clarify the judgements that are made when going from evidence to a decision:

1. What is the balance between the desirable and undesirable impacts of the policy or programme?
2. How confident are we about the likely impacts?
3. How confident are we about the importance of the impacts?
4. What are the resource implications of implementing the policy or programme?

5. Is implementing the policy or programme a priority?

1. What is the balance between the desirable and undesirable impacts of the policy or programme?

The most important consideration that drives a decision, or should drive a decision, about a health policy or programme is whether it does more good than harm. By 'good' we mean all of the advantages of the policy or programme that decision makers consider important and by 'harm' we mean all of the disadvantages. In Article 11 in this series we addressed the construction and interpretation of a balance sheet as a tool to aid judgements about the trade-offs between the desirable and undesirable impacts of a programme or policy [3]. Regardless of whether this is done systematically and transparently (as we would recommend) or in the heads of policymakers, an assessment of this balance underlies any policy decision. When the net benefit (that is the difference between the desirable and undesirable consequences), is large we are more confident about a decision. When the net benefit is small we are less confident (see Box 1).

2. How confident are we about the likely impacts?

In discussing balance sheets [3], we suggested six factors that can lower our confidence in estimates of the impact of a policy or programme. These include assessments of the risk of bias (systematic errors), precision (random errors), consistency of the results across studies, and how directly relevant the evidence is.

Generally, the less confident we are about the likely impacts of a policy or programme the less confident we are when deciding what to do (see Box 2). There are, however exceptions to this. Firstly, we may have so little confidence about the impacts of something that it is easy to decide not to do it.

Secondly, despite little confidence in the benefits of something it may be easy to decide to do something because there is little or no risk of harm, it doesn't cost much and it might do some good [3]. Many types of health information might fall into this category. However, policymakers should be cautious about assumptions that seemingly harmless polices and programmes cannot do harm [8]. Even something as simple as providing health information can, in fact, be deadly [9]. An example of this is advice that was given to mothers in many countries around the world for nearly a half century that babies should sleep on their front. This seemingly harmless advice caused tens of thousands of deaths from sudden infant death syndrome [10].

Thirdly, despite important uncertainty about the likely impacts of a policy or programme, it may be easy to come to a decision that something that is promising should only be done in the context of a well-designed evaluation of its impacts [11].

3. How confident are we about the importance of the impacts?

The net benefit of a policy or programme depends not only on the size of the desirable and undesirable effects (e.g. the difference in the proportion of people who experience a bad

outcome with and without the programme), but also on how important the different outcomes are. For example, drugs with relatively frequent but minor side effects are commonly registered by drug authorities and covered by drug insurance, whereas drugs with rare but serious adverse effects are commonly taken off the market. Clearly serious adverse effects are much more important to patients and policymakers than minor side effects. The same is, of course, true for benefits. For example, governments are often more willing to pay for clinical interventions that reduce the risk of important outcomes, such as death, stroke or heart attack – even when the size of the effect is small – than for interventions with large effects on less important outcomes, such as minor symptoms.

Not uncommonly, the relative importance of different outcomes will vary widely. For example, preferences of women with early breast cancer are highly variable. In a study of women who had completed adjuvant chemotherapy, most considered a 3% increase in survival rates sufficient to make adjuvant chemotherapy worthwhile, but 16 to 32% did not [12]. In a series of experiments using hypothetical scenarios, the relative importance of the benefits and downsides of three different treatments (statins, antibiotics for sore throat, and antihypertensive medication) participants' values were found to vary substantially [13-15]. As anticipated, there was a clear association between how important the different outcomes were to the participants and the decisions they made about treatment.

When clinical decisions are sensitive to patients' values (i.e. how important they think the estimated advantages and disadvantages are), the choice of treatment should belong to the patients. Policies relating to how these interventions are delivered should take account of this. For example, it is inappropriate to use the provision of a preference-sensitive clinical management strategy as a quality indicator.

In practice, medical opinion rather than patient preferences tend to dominate decisions that are preference-sensitive [16]. Policies to promote shared decision making (collaboration between patients and caregivers to come to an agreement about a healthcare decision) or well-informed decisions by patients could help to address this problem. It also might save resources by reducing the delivery of services that are driven more by supply than by patient preferences [17].

When there is disagreement amongst policymakers and stakeholders about whether the benefits of a policy or programme are worth the down sides, differences in their judgements about the relative importance of the advantages and disadvantages may help to understand and, in some cases, resolve disagreements. It may be helpful to first explore the extent to which there is agreement on the underlying evidence (ideally, summarised in a balance sheet). This can help to clarify the extent to which disagreements are due to different judgements about the evidence versus different judgements about the relative importance of different types of consequences.

In addition to the need to clarify judgements about the relative importance of the advantages and disadvantages of policy options, it may sometimes help to recognise that people have different attitudes towards risk (Box 3). Whereas some people are risk averse, others are risk takers, including both policymakers and the general public. This can influence individual decisions, for example about health insurance, the impacts of policies (e.g. targeted at extending health insurance coverage) [18], and policymakers personal views about a policy or programme.

In addition, policymakers may have different attitudes towards uncertainty, which can also help to understand disagreements (Box 4), although it may be hard to distinguish attitudes towards uncertainty from attitudes towards risk and differences in values.

4. What are the resource implications of implementing the policy or programme?

The resource implications (costs or savings) of a policy or programme can (and should) be considered along with other impacts as a consequence of the policy or programme. We address considerations about resource use in Article 10 in this series [19]. Generally, the more it would cost to implement a policy or programme, the less confident we are likely to be about a decision. The greater the cost, the more likely we are to want larger net benefits (excluding costs), impacts on important outcomes, and compelling evidence.

5. Is implementing the policy or programme a priority?

Even when we are confident about the impacts of a policy or programme, it may not be a priority. The extent to which we are confident is a critical factor for deciding on what to do and the extent to which doing something is a priority. However, other factors may determine whether or not a policy or programme is a priority and, ultimately, warrants being implemented. The following factors are incorporated in the considerations addressed above, but may sometimes be considered independently as criteria for setting priorities:

- How serious the problem is – the more serious a problem is, the more likely it is that a policy or programme that addresses the problem will be a priority
- The number of people that are affected by the problem – the more people that are affected, the more likely it is that a policy or programme that addresses the problem will be a priority
- Benefits – the large the benefit, the more likely it is that a policy or programme will be a priority
- Adverse effects – the greater the risk of undesirable effects, the less likely it is that a policy or programme will be a priority
- Resource use (costs) – the greater the cost, the less likely it is that a policy or programme will be a priority
- Cost-effectiveness – the lower the cost per unit of benefit, the more likely it is that a policy or programme will be a priority
- Impacts on equity – policies or programmes that reduce inequities may be more of a priority than ones that do not (or ones that increase inequities)
- Political acceptability – the more politically acceptable a policy or programme is, the more likely it is to be a priority
- Public acceptability – the more public acceptable a policy or programme is, the more likely it is to be a priority

Decisions about priorities should rest on shared criteria or reasoning, be publicly accessible, be possible to appeal in light of considerations that stakeholders may raise, and should be enforced (to ensure that the first three conditions are met) [7]. When criteria such as the above are used implicitly rather than implicitly, it is difficult to judge whether the criteria being used or the decisions were appropriate (Box 5).

Resources

Useful documents and further reading

- Guyatt GH, Oxman AD, Kunz R, Falck-Ytter, Vist GE, Liberati A, et al. Going from evidence to recommendations. BMJ 2008; 336:1049-51.

- Guyatt GH, Oxman AD, Kunz R, Jaeschke R, Helfand M, Vist GE, Schunemann HJ, and the GRADE Working Group. Incorporating considerations of resource use. BMJ 2008; 336:1170-3.

Links to websites

- GRADE Working Group http://www.gradeworkinggroup.org/

Box 1. An example of considering the net benefit of a programme: magnesium sulphate for eclampsia and for non-severe pre-eclampsia

Eclampsia is a serious condition characterised by convulsions or seizures brought on by seriously high blood pressure in pregnancy (pre-eclampsia). Most people would agree about wanting to ensure that women with eclampsia are treated with magnesium sulphate. Magnesium sulphate is inexpensive, has minor side-effects and reduces mortality from 38 to 23 per 1000 women, as well as reducing the recurrence of convulsions. The net benefit is large. On the other hand, the net benefit of treating women with non-severe pre-eclampsia with magnesium sulphate is much smaller, making a decision more difficult. Women with non-severe pre-eclampsia have a low risk of eclampsia (15 per 1000) that is reduced to 6 per 1000 with magnesium sulphate. Given the costs of administering magnesium sulphate to these women and the burden on both the women and the health system, different health systems and different people within the same health system might disagree about the net benefit of ensuring that women with non-severe pre-eclampsia are treated with magnesium sulphate.

Box 2. An example of considering how confident we are about the likely impacts of a policy: Avian influenza

The spread of avian influenza A (H5N1) virus to poultry and wild birds increased the worldwide threat of human infections from the H5N1 virus. This forced governments and clinicians to decide how to deal with this threat and, in a small number of sporadic cases, how to manage infected patients. Because there was no prior experience with this virus, there was almost no direct evidence of the effects of oseltamivir, or other pharmacological agents. Thus, decisions about oseltamivir were made on the basis of trials with patients with seasonal influenza and laboratory studies. Although this was frequently not communicated clearly, estimates of the effects of oseltamivir on avian influenza based on its effects on seasonal influenza and laboratory studies are very uncertain. This is because the two conditions are quite different, little is known about avian influenza in humans, and it is not possible to confidently estimate the impacts of oseltamivir on avian influenza based on studies of seasonal influenza or laboratory studies. Thus, governments made different decisions about oseltamivir (and other plans) for the prevention and treatment of avian influenza based on very low quality evidence [20].

The cost of reviewing the evidence for decisions about worldwide problems, such as avian influenza, can be prohibitively high for low and middle-income countries. It can also be wasteful for high-income countries to duplicate this process unnecessarily [21]. While many countries developed plans for avian influenza [22], it is unclear what the basis was for many of the decisions, the extent to which uncertainty was acknowledged or how that affected decisions. At least some analyses used to inform decisions did not adequately inform policymakers about the uncertainty of the evidence for the decisions they needed to make. In a situation such as this, the development of rapid advice using a robust and transparent process that simplifies adaptation to specific settings could provide an important service to low and middle-income countries, save resources, and help to ensure that important uncertainties are transparent to those responsible for making decisions.

Box 3. An example of the impact of values and attitudes towards risk on a health policy: extending health insurance coverage

In the United States and other countries that do not have universal coverage, policies to extend private or public health insurance coverage to uninsured people often have mixed results, with many individuals not taking up offers of health insurance. Knowing how people value health insurance and their attitudes towards risk can help to craft policies [18]. For example, mandated approaches – such as an employer mandate or state mandated benefits - may have unintended consequences for individuals who don't value health insurance. On the other hand, voluntary approaches may require creating financial incentives (targeted at people who do not think coverage is worth the cost) and additional information on the value of health insurance to individuals (for risk takers and people who believe they don’t need health insurance, as well as for those who do not think coverage is worth the cost).

Box 4. An example of attitudes towards uncertainty in relationship to a health policy decision: human papillomavirus (HPV) vaccination

Policymakers in middle and high-income countries have struggled with decisions about HPV vaccination, in part because of uncertainty about the impacts of different HPV vaccination policies. Estimates of the impacts of vaccination on the incidence of cervical cancer or death from cervical cancer are uncertain [23-25]. This is because the evidence of the effectiveness of HPV vaccination is indirect. Although the trials were large and well designed, they used surrogate outcomes (lesions that are precursors of cervical cancer), in different populations (girls that are older than the age at which HPV vaccination is commonly recommended), that were followed up for only five years (so it is uncertain what longer term effects will be). In this case there is a risk of not vaccinating (and not preventing cervical cancer that could be prevented if HPV vaccination is effective) and a risk of vaccinating (potentially causing unintended long-term adverse effects and wasting resources, if it is not effective).

Even after agreeing about the uncertainty of the impacts of HPV vaccination and the relative importance of different outcomes, people may still disagree about whether HPV vaccination should be recommended and covered by health insurance. One reason for this is different attitudes about uncertainty. Some people believe that HPV vaccination should be recommended routinely and publicly funded despite the cost and the uncertainty about its benefits and potential long-term adverse effects. Others feel that it should not be recommended routinely or publicly funded until there is better evidence.

Box 5. An example of non-systematic and non-transparent criteria and judgements regarding priorities for a drug benefit programme

The Norwegian government provides drug insurance as a part of the National Insurance Scheme, which is overseen by the National Insurance Administration (NIA) [26]. Up until 2000 the NIA was responsible for evaluating applications to add new drugs to the drug benefit programme. A review of NIA documents for applications in the 1990s found eight factors that possibly influenced decisions: the treatment effect, side effects, cost-effectiveness, total costs to the NIA, control of (inappropriate) use of the drug (and expenses), administrative constraints, seriousness of the condition, and equity [27]. There was rarely an explicit written evaluation for any of the factors and it is not clear to what extent most of the factors were considered for most of the applications. Because the assessments were neither systematic nor transparent, it is difficult to judge whether the criteria that were being used or the decisions were appropriate.

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