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Qualitative analysis – expert testimony on the best methods measuring cost-effectiveness of chronic diseases

WP 4: Forming a consensus on methodology for measuring cost-effectiveness of interventions for chronic diseases

EConDA
Economics of Chronic Diseases

This report arises from the EConDA project which has received funding from the European Union, in the framework of the Health Programme. Contract number: 2012 12 13

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Executive summary

The burden of chronic diseases is rising across the European Union. This is not only driven by increases in life expectancy but also by changes in risk factors. Whilst smoking rates are falling in some countries, in many they are not and importantly not amongst the most disadvantaged populations with already increased risks for chronic diseases. As a result there are greater disparities in health. Other risk factors such as obesity are rising sharply across the EU placing an increasing burden on countries' health systems. This is occurring in a climate of economic austerity creating a greater imperative to justify health interventions to both prevent and address existing cases on the basis of cost-effectiveness.

This study takes a qualitative approach to understanding methods for measuring the cost-effectiveness of interventions that prevent and treat chronic diseases. Gaining expert testimony through qualitative interviews is important in deepening our understanding of the methods used, why they are chosen and how they can be developed. Also, the approach aims to identify the specificities in cost-effectiveness of chronic diseases as opposed to other disease areas.

For this purpose, Thematic Framework Analysis was carried out. Three major themes emerged: 1. 'determining the exact method of cost-effectiveness analysis', 2. 'data limitations', 3. 'research recommendations'. Various subthemes emerged for theme 1 and 3, in particular. For theme 1 these were monetary valuation, country specificity, non-communicable disease characteristics and comparisons across disease areas. Thus, methods were chosen in order to place a monetary value on the outcome, or in accordance with country specific reasons to choose one method over the other (e.g. politics, guidelines or, health system). Cost-utility analysis (CUA) was mentioned to be most suited within the broader set of cost-effectiveness methods for non-communicable diseases and enables comparison across diseases because it uses the standard QALY. A general preference was noticed for Markov modelling.

Despite most experts preferring and employing cost-utility analysis and/or Markov modelling, a number of limitations were highlighted. A mixed-methods approach was certainly also deemed acceptable. However, there was no real consensus over how methods should be mixed – whether it is cost-utility with cost-effectiveness per se (clinical outcomes) or cost-benefit, or all three.

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Six subthemes emerged from theme 3, all hinting at research recommendations which will potentially be a useful springboard for discussion about reaching a consensus on the best method for measuring cost-effectiveness interventions for chronic diseases.

Encouragingly, experts believed that it was feasible to reach a consensus. Citing one of the experts: 'I think reaching a consensus...in a standard approach, developing guidelines....is quite possible.

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Introduction

Widespread austerity measures across Europe has resulted in healthcare resources being scarcer than ever, while a changing demography has seen life expectancy increases, and with it, an upsurge in years lived with a (chronic) disease. More people than ever are living with chronic diseases, imposing high costs on the health-care system. Notably, potential interventions are competing for the allocation of scarce resources more than ever. Therefore, knowing which interventions are both the most effective and cost-effective to prevent and treat chronic disease is imperative.

There are three main approaches to measuring cost-effectiveness (CE): cost-effectiveness analysis per se (CEA) compares the relative costs and outcomes of two or more courses of action, while cost-benefit analysis (CBA) assigns a monetary value to the outcome measure. Cost-utility analysis (CUA) estimates the ratio between the cost of a health-related interventions and the benefit it produces in terms of the number of years lived in full health by the beneficiaries; i.e., outcomes are measured in Quality Adjusted Life Years (QALYs). Measurement of cost-effectiveness has a long history. Despite this, there is no consensus over how best to measure cost-effectiveness in health or for chronic diseases per se.

Expert testimony is useful to enrich our understanding of how CE is measured, the limitations of measurements and how the methods can be improved.

The aim of this study was therefore to carry out a qualitative analysis to explore the methods used to assess cost-effectiveness of interventions for chronic diseases. This study uses thematic framework analyses (Ritchie and Lewis, 2003) to categorise expert health economists' views of the best methods for measuring cost-effectiveness. The methods undertaken are presented in appendix 1.

Expert information

Twelve experts were interviewed, who are working from a range of countries: Russia (n=1), Australia (n=1), UK (n=4), Portugal (n=1), France (n=1), Belgium (n=1), Netherlands (n=1), Germany (n=1) and Spain (n=1) though often were citizens originated from other countries. Experts were mostly academic health economists working at universities (n=9), although one expert was from the OECD, one is a medical health professional and one a health economic consultant. All interviews were conducted in English and are anonymised with an ID from 1 to 12.

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Analysis and discussion

Three themes emerged from the framework analysis: 1) Determining a method of cost-effectiveness 2) Data limitations 3) Research recommendations. There was no absolute consensus over which method was best, although all interviewees said that CEA and CUA were used most frequently. The framework is presented in Table 1. The thematic framework is presented in Appendix 2.

Emerging themes

Chart 1: Determining a method of cost-effectiveness

Most experts described context specificity as important when deciding which method of cost-effectiveness analysis to use. One expert summed this up, '...maybe there are different views at the start...but they are mainly related to different situations' (ID 4). This theme was divided into five subthemes.

Monetary

If the aim of the cost-effectiveness exercise is to decide where best to allocate resources then 'in the UK and in some other countries, for the purposes of deciding where to spend money they use...CUA' (ID 4). In the UK, NICE guidelines recommend the use of CUA for these purposes. Another approach - cost-of-illness-DALY's - are used to ascertain the overall monetary value to provide an overall picture of economic impact of the disease: '.....in the Pacific regions cost-of-illness-DALYs approach is used….those countries want to be able to allocate limited resources' (ID2).

Country specificity

Factors within and between countries were important for the choice of method used, but were also the cause of difficulties in comparing across countries.

The political or healthcare system in the country was one within country factor influencing the choice of method: 'It depends on politics and how the healthcare system operating within the country of interest is managed' (ID3); 'Some government bodies, they prefer to use some measures

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that can be used commonly' (ID4), 'QALYs...might be considered to be the most used in the UK whereas in developing countries one might argue that....CBA, their willingness to pay...might be considered most popular...so its context specific' (ID8). Guidelines exist in some countries and this explained why some methods were chosen over others: 'CBA is less widely used in the Netherlands at least, net benefits are commonly not calculated because it's not mentioned in the Dutch guidelines for performing CE studies' (ID10); in the UK...most of our analysis is supposed to feed into NICE guideline development and I think that specifies quite clearly that CUA is a preferred' (ID8).
A cross-country comparison of costs was viewed as difficult, 'there is a problem if you compare cost effectiveness levels across different countries…..because they could have been estimated differently… using different populations.....so it's quite difficult to apply CE results from one country to another country, then you probably need to conduct CEA in that jurisdiction too which can be quite expensive' (ID 4).

Comparing physical activity interventions across countries was used as an example to illustrate difficulties: '…there is a specific interest in interventions for letting people get more exercise by biking to work, walking more…playing sports….and again the result you are going to get from these kind of evaluations are very context specific so results may be very different in the UK to say Russia or Sweden or Switzerland' (ID 4).

Non-communicable diseases

When measuring cost-effectiveness for non-communicable disease interventions the method to choose was said to be CUA: 'CUA is most apt for NCDs' (ID2); 'for NCDs I think CUA with QALY because interventions that target risk factors of coronary diseases they can potentially affect both the length of life and the QoL' (ID4); 'Cost Utility Analysis...because it can be used for both mortality and morbidity and where Health related Quality of life is an outcome' (ID2).

Comparing across disease areas

Cost-utility analysis using the QALY was used because it enabled comparison across disease areas, where other methods cannot: 'with CEA it's quite difficult to compare the value of different interventions if their benefits are expressed in different units….' (ID5) 'if you do CEA and focus your attention on a certain health area it is very difficult to make a comparison with a completely different disease area' (ID4). However, 'CUA can be used to compare across various diseases and interventions within and across disease areas e.g. it might be important to compare the benefit of

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interventions in heart disease with that of cancer. Also, in the case of different healthcare outcome matrix measures like DALYs and QALYs gained can be compared' (ID2), 'the QALY is really capable of comparing interventions in terms of health benefits across different chronic diseases like diabetes, cancer, CVD....I think you should always compare to the alternative' (ID7).

CUA was often the choice of governments because: 'the government and public bodies...in charge of the health budget are primarily interested in being able to compare interventions across various disease areas...' (ID4).
The type of model chosen to measure cost-effectiveness can be influenced by the 'relationship of one disease with other diseases…for example 'if you have one chronic disease that has a relation with other chronic diseases e.g. CVD, diabetes, obesity it is much better to use discreet event simulation modelling, but for e.g. with Parkinson's disease, it does not have many relationships with other diseases maybe with a Markov model it is better because you can find more parameters for the model, more information than with a discreet event simulation model' (ID11).

Prevention or treatment

The choice of method was also influenced by the type of intervention tested – whether it was prevention or treatment or both: 'If you are talking about evaluation of prevention or healthcare generally because clearly there is a difference….if the focus is on prevention, then CEA is very helpful' but 'you need to compare the outcomes of a prevention programme to those of standard treatments in the healthcare domain so adding a cost-benefit component.....is extremely important' (ID5).

The QALY is often used as an outcome measure in cost-effectiveness, however, one expert suggested that this may not be appropriate for the healthcare sector because 'the QoL measures which are used for the purposes of constructing the QALY can be quite insensitive to therapeutic interventions which are promoted by the private sector' (ID 4). Instead, '..in the private sector CEA [as opposed to CUA with QALYS] are used quite a lot...because the private sector is interested in promoting their own drugs or medical devices, and sometimes its quite difficult to show the value if you use QALY...for example the impact on life expectancy' (ID4).

CBA was said to be essential in healthcare because 'this is how we get interventions embedded into health systems…that would prove an enabler to making interventions happen, making strategic change....that is really key' (ID9).

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Modelling strategies were said to be necessary in prevention because '….trials are very difficult if not impossible and you need alternative approaches for supporting an economic evaluation when you're trying to assess the outcomes and the value of prevention so modelling is clearly a key addition to CEA frameworks for prevention' (ID 5).

Chart 2: Data limitations

A number of data limitations were described with aspects of the methods used in cost-effectiveness. Data limitations were often described as hindering the process.

For example, although many strengths were reported 'CUA [is best] because it has clear advantages compared with other methods …with CUA you use complex effectiveness criteria, QALY, QoL years saved,…[however] to receive such parameters it is necessary to get prospective data which is very difficult, it's very limited in our countries [Russia]' (ID1).

Carrying out modelling exercises is also limited by the quality of data available: 'it demands the use of data, in the absence of data we end up making assumptions, for example an obesity intervention is usually over a 12 wk period and we try to build on how effective this is in the lifetime of the individual... something that we really don't know' (ID8); however, despite these assumptions modelling is a useful tool: 'for modelling you can find the data from the literature… you try to complement the individual patient data with the modelling... we build a model to replicate the individual patient data from follow-up...once they have that they extrapolate the results to expand over some time....so I think that is the best approach' (ID11).

Choice of modelling methods is dependent on the type of data available: 'you choose between Markov model and discreet event simulation model, with Markov it's easier to find the parameters in the literature, you can estimate probabilities from sample sizes...for discreet estimation modelling there are many problems to estimate the time….there are not many methodologies available to estimate some kind of data...' (ID11).

Chart 3: Research recommendations

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This chart builds on the previous by describing what needs to be considered in terms of methods for economic evaluation and how methods should be improved in the future. This may help form the basis for guidelines on the best method for measuring cost-effectiveness.

Intersectoral comparison/Societal perspective

Taking a societal perspective was an important recommendation for future economic evaluations, because 'I don't [think] we do it at the moment really; it would be lovely to provide a wider economic impact…a societal approach' (ID9). In fact one expert said: 'taking a societal costs perspective is a gold standard' (ID2)

A societal perspective looks beyond healthcare and enables an intersectoral comparison: 'It's preferable to not have a very narrow focus but to have a societal perspective in this kind of evaluation because that can have a big impact upon the number of outcomes independent of individual health, it can have outcomes for the health system as a whole' (ID4).

CBA is the method of choice for enabling a societal perspective to be taken: 'One of the strengths of CBA is that it takes a societal perspective into account….' (ID2); 'CBA is the only one that allows intersectoral comparison...so even though very few people do it…. ideally you should have CBA...alongside CEA' (ID4). 'I myself try to do as broad as possible, I think if you try to test the impact of whatever intervention you should try to measure all of the effects' (ID7).

Intersectoral comparison was described by most of the experts as an important consideration in cost-effectiveness analysis: 'adding a cost-benefit component ...is extremely important because many of the prevention strategies that the government's may want to consider are clearly not just within the healthcare sector but....in other sectors of government ....transportation, urban planning...education' (ID5).

Being able to compare interventions that go beyond health was deemed important: 'need to make sure that outputs of economic analysis are comparable with interventions that take place in those sectors and CBA is the only one that allows intersectoral comparison' (ID5).

'There needs to be a new method…that encompasses relevant non-health outcomes...a new branch that creates interventions in e.g. obesity outside the healthcare sector per se i.e. food, transport, air

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pollution etc using CBA. It is challenging to integrate this with health outcomes. Therefore, the need is to figure out how to combine CBA with CEA' (ID2).

Combining approaches

Combining methods emerged as an important recommendation for future work. However, there seemed a differences of opinion over which methods should be combined and this most probably is determined by many of the factors in chart 1.

Combining CBA and CEA: 'the starting point is always what is now a classical economic analysis based on cost-effectiveness evaluations...possibly with the addition of a cost-benefit component looking at prevention strategies and use that as a starting point, then on top of that you can use all sorts of complementary evaluation....figure out how to combine CBA with CEA…ideally you should have CBA...alongside CE' (ID5).

One participant suggested building upon CUA: 'A CUA which used QALYs - I think if you were realistically going to extend this then you would add in a dimension of DALYs and years of potential life lost because I think those 3 methods together give you quite a good economic model' (ID9).
CUA and Cost-Conseqence Analysis (CCA): 'I've always been a proponent of a mixed-methods approach, our hands our tied by the NICE [guidelines] which is a CUA, but….I think we need CUA+CCA whereby we will be able to satisfy NICE by using the QALY and at the same time get an indication of other outcomes which might not have enough robust quantifiable evidence to allow them to be presented in a QALY, presented alongside costs, for e.g. if you've got an intervention, it will be difficult for a local commissioner to understand what a QALY transfers into in his local setting ...they can at least understand where an intervention leads to a reduction in hospital days' (ID8).

All three approaches: Another expert suggesting combining all three approaches within a modelling framework: 'a combination… good long clinical trials in combination with real life outcome data from patient registries together in a modelling framework ...when you have the model you can do all of them [CBA, CUA, CEA]' (ID10).

Modelling: 'One thing I think we totally agree on is the need for modelling tools that help predict the large scale consequences of strategies that can be applied at the national and regional level which cannot otherwise be predicted by RCTs' (ID4).

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Cost-effectiveness ratios

‘you should not really change the current cost-effectiveness analysis approaches to try and incorporate equity concerns into the existing cost-effectiveness ….but you should use an approach that produces cost effectiveness ratios... then separately produce evidence of impact or inequalities that decision makers can use alongside CE ratios in which a decision on the appropriateness and efficiency of a particular intervention...’ (ID5).

Standardised terminology

A couple of experts highlighted the need to standardised the terminology used: ‘there's lots of terminology which….people might sometimes say that they do a cost-effectiveness analysis but in another instance they say they do a cost-benefit analysis but both instances they would express health benefits and QALYs, they might give a different label to it which creates confusion, and the same goes also for the sort of perspective' (ID7).

‘The first question is what do you actually want to measure? You need to clearly state the problem that you are trying to get an answer to….the economic evaluation should support a decision maker ....' (ID7).

A standard framework

One expert suggested that a framework would be useful: ‘I think there is a lot that we can standardise in terms of the approach … so there is a standard framework within which we can devise a method and an approach for evaluating prevention interventions not all of the components of that framework have been sufficiently developed...' (ID5).

Modelling both public health and clinical interventions

'It would be good to see the interplay between PH interventions and more clinical interventions and to perhaps model the efficacy in terms of outcomes of the extremes of doing one or the other...take MI for e.g. would it be enough to have a legislative smoking cessation service and what impact would that give us or at the other extreme you wouldn't have anything in risk factor prevention and just do illness management systems…. then there’s a huge grey area in between and what sort of models do you have to drive your health system to best spend its resource' (ID9).

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Conclusion

In general, experts described cost-utility analysis and Markov modelling as the most widely used approach. However, there were a number of factors that needed to be considered when making a choice over a method, as well as a number of limitations of the methods. There was no clear consensus over which one was best, and there was a general view that a mixed method approach was useful. Also, 'there is a need to improve all measures and make them more precise' (ID 2). QALYs were the most frequently used outcome measure, although all experts noted limitations with this method. One expert pointed out that: '...if you abandon the QALY what would you do then? How would you then make decisions...? It can always be improved....if you abandon the current state of the art to what then do we resort?' (ID7). Therefore there should be discussion relating to improving current methods, rather than creating new ones which are established 'it's about what elements to add to the existing framework' (ID 5).

Equally, it is important to be mindful that it is not always possible to get a reliable measurement of an individuals' health state in terms of utility: 'there will never be a gold standard that is objective, always there will be errors….don't believe there is a truth out there that you can measure…at the end you still have to make the decisions according to the standard of which as a society we agree upon' (ID8). 'Once the special requirements of specific interventions are taken into account I think reaching a consensus...in a standard approach, developing guidelines....is quite possible (ID5).

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Appendices

Appendix 1: Methods

Expert information

It is recommended that interviews are carried out until thematic saturation is reached. Initially, ten participants were contacted by email to take part. Seven replied agreeing to take part and interviews were carried out. A further seven were contacted and four replied agreeing to take part. Therefore, in total twelve experts were interviewed, who are working from a range of countries: Russia (n=1), Australia (n=1), UK (n=4), Portugal (n=1), France (n=1), Belgium (n=1), Netherlands (n=1), Germany (n=1) and Spain (n=1) though often were citizens originated from other countries. Experts were mostly academic health economists working at universities (n=9), although one expert was from the OECD, one is a medical health professional and one a health economic consultant. All interviews were conducted in English.

Semi-structured interview

Semi-structured interviews enables the interview to be steered by the interviewer to gather data related to the study aims and for comparison across interviews to be made, not always possible using an unstructured interview design but allows more flexibility than a structured design (Ritchie and Lewis, 2003). Priority was given to asking the most neutral and open questions possible. Prompts were used if needed, and the topic guide used flexibly depending upon participant responses: questions were changed, asked in a different order; new ones added or left out. General prompts such as 'can you tell me more' were used (Arthur & Nazroo, 2003).

Face-to-face interviews have the advantage of interpreting social cues (e.g. body language) of the participant, telephone interviews are increasingly being used because they are efficient and cost effective. They also have the advantage of being less intrusive and reactive than face-to-face interviews making the interviewee more comfortable and in turn encouraging more open and honest descriptions (Opdenakker, 2006). Therefore, telephone interviews are used in the present study. A topic guide was developed to form a semi-structured interview (appendix 1).

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Analysis

Thematic Framework Analyses (Ritchie and Lewis, 2003) was carried out to categorise experts' perceptions into themes and sub-themes. This method is a useful way of managing, ordering and filtering the large amounts of interview data produced more easily. It uses a thematic approach, but analysis involves extracting themes both from the data and research questions through familiarisation of the transcripts; Identification of a thematic framework by indexing key issues into themes and subthemes; Indexing - applying the thematic framework systematically to the data; and Charting by creating a series of matrices that each contains one theme.

Text was coded more than once if it conveyed more than one meaning, and coded extracts included in as many different themes as was relevant. Surrounding data was included in the code so that context was not lost. Initial codes were then arranged loosely into a broader set of key themes and sub-themes to form a thematic framework. Once the matrices were constructed, thematic analysis was applied for each theme across all respondents. Refinement of codes and themes occurred throughout the analytic process as the data becomes more familiar.

In order to check validity of the codes and themes two researchers (LW & TM) independently rated two transcripts using the thematic framework. This helped to ensure agreement of interpretation and enabled greater objectivity. Reflexivity is also viewed as key to improving reliability and validity. This posits that the characteristics of the researcher (age, sex, ethnicity) should be described which might influence the research and analyses. Thus, acknowledgment that some element of bias is inherent in qualitative research should be made. The use of more than one researcher allows for consistency and reliability in interpretation of data (Perry, 1994; Daly et al, 1992)

Appendix 2: Thematic framework

Table 1 describes the thematic framework that emerged from the interviews. Three key themes emerged relating to aspects of measuring cost-effectiveness (CE) in health and these are described as: 1) Determining a method of cost-effectiveness 2) Data limitations 3) Research recommendations. The following section describes and discusses these themes.

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Table 1: Thematic framework

Chart 1: Determining a method of cost-effectiveness
Monetary
Country specificity
Non-communicable diseases
Comparing across disease areas
Chart 2: Data limitations
Chart 3: Research recommendations
Intersectoral comparison/societal perspective
Combining approaches
Cost-effectiveness ratios
Standardised terminology
A standard framework
Modelling both public health and clinical interventions

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Appendix 3: Topic guide

Project description:

Hi,…

Many thanks for taking the time to speak to me today. I thought I would give you a brief description of the project first then some general questions to discuss.

So, as you may know, the aim of the EConDA project is to aid EU Member States to implement more cost-effective policies to improve chronic disease prevention.
One of the specific objectives is to seek consensus among relevant experts on the methodology for measuring cost-effectiveness of interventions to prevent, screen and treat chronic diseases, and that is the aim of this interview - to gather your views on the best methods for assessing cost-effectiveness of four chronic diseases – CHD, COPD, type 2 diabetes and chronic kidney disease.
If we do not reach a consensus, then we will aim for a set of guidelines of 'best practice'.
The interview is semi-structured so feel free to discuss the issues more pertinent to you in greater depth.

Questions for interviews are:

1. Which method of economic evaluation (CUA, CBA, CEA…) is used most widely?

2. What do you think is missing from these evaluation methods?

3. Strengths and weaknesses (limitations) of these methods.

4. What are the most appropriate methods and why? Can you give me some examples?

5. Which method is the best for NCD evaluation?

6. Which methods do you use most often and are there reasons in particular why you have chosen these?

7. How is it best to capture indirect costs do you think?

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