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Work Package 4: Consensus meeting

Establishing a consensus on the best methods for measuring the cost-effectiveness of interventions to prevent, screen and treat chronic diseases

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EConDA
Economics of Chronic Diseases

This report arises from the project EConDA which has received funding from the European Union in the framework of the Health Programme.

Executive summary

EConDA (Economics of Chronic Diseases) is a European Union co-funded project that aims to reach a consensus amongst experts over the best way to measure cost-effectiveness (CE) of interventions for chronic diseases. It will use this consensus to model the cost-effectiveness of interventions to prevent, screen and treat four major chronic diseases (Type 2 Diabetes (T2D), Coronary Heart Disease (CHD), Chronic Obstructive Pulmonary Disease (COPD) and Chronic Kidney Disease (CKD)).

As background to an expert meeting that aimed to reach a consensus, two reports were written: 1) A literature review of the cost-effectiveness studies for each of the four diseases was carried out. This reviewed 153 studies in total. Twenty-nine studies for T2D, 84 for CHD, 22 for COPD, and 18 for CKD. For T2D, various interventions such as Intensive Lifestyle Management programmes, smoking cessation, metformin, testing glucose or albumin levels proved to be cost-effective 2) A qualitative study complemented this review. It used thematic framework analysis to gain a more in-depth understanding of the methods. Three major themes emerged: 1. 'Determining a method of cost-effectiveness', 2. 'data limitations', 3. 'research recommendations'.

These reports were used as a springboard for discussion in an expert consensus meeting in December 2013. Some consensus was gained over the methods that should be used for the EConDA modelling:

- different ICERs should be used: life-years gained; DALYs; QALYs; number of cases, direct costs and indirect costs
- a societal perspective should be taken where possible and this should be country specific.
- it was agreed that there should be no standardised CE cut-off points as comparing CE is problematic. However, it was agreed that comparing cost per QALY could be useful.
- cost per QALY should be used to compare across countries, though there was agreement that parameters should be country-specific e.g. the discount rate, currency, utility measures, way of calculating the societal perspective etc.
- use of a discreet event simulation model should be used

Data availability was highlighted as a key limitation to the quality of the modelling and confidence intervals should be presented with the outputs.

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Background

Healthcare budgets are tightening across Europe, so investing in cost-effective interventions is more important than ever. Chronic diseases are responsible for the vast proportion of morbidity and mortality across the World Health Organization (WHO) European Region and are therefore of special interest to policy makers tasked with preventing disease and allocating healthcare resources. To ensure these decisions are as efficient and effective as possible, it is vital that the cost-effectiveness of interventions to prevent and treat chronic diseases in a population is well measured. Presently there is inconsistency in the methods used making comparisons between studies difficult. Conclusions drawn in one setting may not be applicable elsewhere. Before research can move forward, it is important to seek a consensus on the best measures of cost-effectiveness of chronic disease interventions.

Work package 4 of the European Union funded 'EConDA' (Economics of Chronic Diseases) project aimed to tackle this need by building a consensus on the best method for measuring cost-effectiveness of chronic diseases and asking how best to model the cost-effectiveness of interventions to prevent, screen and treat four major chronic diseases (coronary heart disease, type 2 diabetes, chronic obstructive pulmonary disease and chronic kidney disease). This work package is made up of three parts: 1) a literature review of articles measuring the cost-effectiveness of interventions for each of the four diseases, 2) a complementary qualitative study to gain a more in-depth understanding of the most effective and feasible methods for measuring cost-effectiveness as well as to uncover the best ways of measuring indirect costs and the future needs in terms of research in this area, 3) consensus meeting.

This report outlines the outcome of part three of work package 4, the consensus meeting. The meeting built on the reports produced in parts one and two. This report serves to inform the modelling that will be carried out in work packages 5-7 (econdaproject.eu).

The consensus meeting considered the following questions:

- What cost-effectiveness outcomes should EConDA model?
- How do we measure direct and indirect costs?
- How do we best measure a societal perspective?
- Should there be a cut-off point for CE and if so, what should this be?
- Can we agree on an appropriate discount rate?
- How do we best compare across countries?

- What are the ways forward and new methods beyond what currently exists?

Aim of the meeting

The aim of the meeting was to:

> Bring together a range of health economy experts from EConDA target countries and from leading international organisations to reach a consensus on the best ways for measuring cost-effectiveness of interventions to prevent, screen and treat chronic diseases.

Invited participants

Participants invited to attend the meeting were identified by the EConDA team and collaborators. Experts from the OECD, WHO, and European Commission were invited to present the approach of their respective organisations in relation to measuring cost-effectiveness as well as participate in the meeting's aim.

Health economists who had expertise in a particular disease area or country were also invited to participate. This breadth of expertise helped ensure that a range of issues were captured.

15 participants attended, including 5 members of the EConDA team and are listed in the Agenda in Appendix 1.

Meeting outline

Format of the meeting

The agenda of the meeting is presented in appendix 1. Ahead of the meeting, participants were sent:

- a review paper of the cost-effectiveness of interventions to prevent, screen and treat four major chronic diseases: coronary heart disease (CHD), type 2 diabetes (T2DM), chronic obstructive pulmonary disease (COPD) and chronic kidney disease (CKD) (appendix 2)
- a qualitative study analysis expert views on cost-effectiveness (appendix 3)

These documents served as a springboard for discussion during the meeting.

Representatives from the OECD, European Commission and WHO were asked to present their opinion, but not necessarily that of the organisation, regarding the strengths and weaknesses of cost-effectiveness methods from the point of view of their organisation.

The meeting was held over two days: the afternoon of 10 December and the morning of 11 December 2013.

Meeting opening

Susanne Logstrup opened the meeting with an introduction to the EConDA project and the aims of the meeting. Laura Webber summarised the reviews and comments were made by the floor.

In general, it was stressed that economic evaluation includes only two perspectives: cost-benefit analysis (CBA) and cost-effectiveness analysis (CEA). Cost-utility and other recent forms of economic evaluations are effectively only sub-types of these two approaches.

In addition, the impact of interventions and of the economic evaluation on equity/equality needed to be considered.

Presentation from representative of the OECD

Dr. Sassi first highlighted a number of questions that the OECD are concerned with:

- What are the relevant effects of prevention programmes (e.g. morbidity/ mortality/ productivity/ impact on public expenditure/distribution of health)?
- How should health be valued in economic terms?
- How can inter-sectoral programmes, involving different government departments be assessed? What outcome measures would be appropriate?
- How should the future impacts of prevention programmes be discounted?
- Could efficient prevention programmes increase health inequalities?

Dr. Sassi outlined strengths and weaknesses of economic analysis:

Strengths

- The value of economic evaluation is in its ability to compare across interventions. Economic evaluations should produce results for a wide range of interventions allowing policy makers to compare them and help them to prioritise allocation of limited resources.

Weaknesses and future requirements of economic analyses

- When reporting economic analysis e.g. cost effectiveness analysis, cost-benefit analysis, it is important that the method used is explicitly stated. Evidence from the review showed that the type of economic analysis used was often unclear.
- Focus should be on interventions not diseases: A cost-of-illness approach is good for publicity because they draw attention to public health issues, but they do not constitute an economic evaluation, they are simply a necessary input to economic evaluation.
- There is a desperate need for epidemiologic data. Modelling is one way to deal with lack of data limitations and to highlight to governments the need to gather surveillance data.
- There is a need for evidence of the effectiveness of interventions on tackling chronic diseases. Better evidence will help to inform policy makers and improve decision making. Further research is required to achieve this. But also EConDA plays a key role by providing population models of health and economic impact.
- Discounting rates differ according to the intervention, but the same discount rate should be applied to costs and benefits.
- The impact of inequalities should be presented separately using a positive, rather than a normative approach.
- CE is often equated with cost-saving. In reality, interventions cost money, but the costs are justified by the effectiveness of an intervention to prevent, diagnose and treat diseases.

Economic evaluation should answer four questions:

1) does prevention improve health?

2) does it reduce health expenditure?

3) does it reduce health inequalities ?

4) is it cost-effective?

Presentation from representative of the European Commission

Dirk van den Steen, Senior Health Economist, European Commission

Summary of presentation

- **EFFECTIVENESS & EFFICACY** A difference between effectiveness and efficacy was highlighted. It is important to keep in mind that some effective treatments may not produce the same results when practiced outside a highly constrained laboratory environment. Therefore, some effective interventions may not be as effective in real life settings.
- Affordability is highly prioritised in the face of an aging society. Health systems may well become more concerned with affordability than with CE.
- In countries with a high prevalence of chronic diseases ("post epidemiologic transition" countries), societal changes (population lifestyle behaviour) arguably have larger effects on population health outcomes than institutional traits of health care systems.
- **INDIRECT COSTS** The method of measurement of productivity losses needs to be agreed upon at a high level. For example, we need to decide if such variables as level of unemployment and retirement age need to be included.
- **MODELLING** is important for competing disease risks and multi-morbidity. However, Markov models may not capture all the aspects of disease aetiology so we need to use a different type of model – e.g. discreet event micro-simulation model which will be used in EConDA.

Presentation from representative of the World Health Organization - Regional office for Europe

João Breda, Programme Manager, Nutrition, Physical Activity and Obesity, World Health Organization.

- **EQUITY –** The new health policy in Europe, Health 2020, has a strong focus on equity. However, there is little emphasis on equity in CE studies. Definitions of well-being and inconsistency of indicators used to define equity are a part of the problem.
- **DECISION MAKING** CE analysis should not form a single basis for decision making, because health should not be valued simply as a cost.
- **MEASUREMENT** Not all risk factors can be effectively measured, and therefore interventions to reduce these risk factors may not appear as cost-effective. For example, it is particularly difficult to accurately measure nutritional intake and physical activity levels. Policy makers

may be reluctant to use data on CE originating from another country as this can be considered contextually flawed.

- **VALUE CREATION** CE should account for value creation, e.g. new businesses tackling overweight/obesity.
- **SIMPLE MODELS** We need to make models simple enough for lay people to understand and avoid the sense of obscurity.

Summary of discussion following the presentations and key messages

The background reviews and presentations were a good springboard for discussion at the meeting.

- **PERSPECTIVE**

Primary prevention at best postpones the incidence of a disease, although both primary and secondary prevention may reduce the demand for health care. Prevention does not necessarily save money and does not eliminate diseases, however to appreciate the full economic impact it is necessary to go beyond the health care perspective.

Sometimes a lifestyle intervention may not be cost-effective compared to pharmaceutical if a narrow health care perspective is taken. However it may be cost-effective if a broader societal perspective is taken.

The effectiveness of public health interventions are difficult to measure in the same way as drugs e.g. using RCTs so they need to be appraised differently. Further, often prevention trials do not have a long enough follow-up to measure their true effectiveness.

In some countries, for example the Netherlands and Portugal, decision-makers have to consider a societal perspective in CE analyses, i.e. include indirect costs. It was also voiced that NICE will recommend this for the UK in the summer, 2014.

Taking a societal perspective in EConDA should be country specific and account for local circumstances. Although this will mean that outcomes are not comparable between countries, decision makers do not make decisions for a few countries, but only for their own country. For example, EUR 20,000 per QALY in one country is not useful for a decision in another country. The

European Commission will need to publish guidance on a European level, before we can use it in our project.

- **INDIRECT COSTS**

What is included in indirect costs is sometimes unclear. Different unemployment rates and retirement ages across Europe makes it difficult to calculate and compare indirect costs across countries.

There should be a logical way of ordering the costs: for example figure 1 in Rappange et al. Pharmacoeconomics 2008;26(10):815-30. The characterization includes direct medical costs, direct non-medical costs, indirect non-medical costs and indirect medical costs (related and unrelated). This last one includes costs in life years gained, which is still a hotly debated topic and which was also discussed during the meeting.

Friction costs: it was mentioned that this method for measuring indirect costs is only used in The Netherlands and Portugal. Other countries either do not use the societal perspective, or use the human capital approach. However, there is some suggestion that NICE will recommend this for the UK in 2014. The way indirect costs are measured is controversial because of the way they are accounted for. Reporting these separately, alongside the main analysis is important.

- **METHOD**

Some advances have been made in statistics and modelling, but none of these changed the foundation of economic methods, therefore established methods (CBA and CEA) are preferable to novel approaches. The principles of economic evaluation (EE) methodology should not change, as it can produce incomparable results. However, evaluation can be adjusted for specific needs and multi-morbidity.

CBA requires the definition of willingness-to-pay for the health outcomes.

- **INEQUALITIES AND EQUITY**

Including inequalities in an economic model can be complicated (see OECD prevention model[1]). It is important to agree how inequalities are measured and what is defined as inequality. Education and income could be used in the EConDA models as proxies for socio-economic

[1] OECD Health Working Papers No. 48 Improving Lifestyles, Tackling Obesity: The Health And Economic Impact Of Prevention Strategies www.who.int/choice/publications/d_OECD_prevention_report.pdf

position, depending on the reimbursement levels of health care systems. Prevention may affect different groups of society in different ways; some prevention interventions have lower uptakes in lower socio-economic groups, though some interventions can be effective across all social strata and ethnicities.

Inequality is not a result of a particular intervention, but rather of the way an intervention is implemented. A question was raised of how we define access to health services or interventions? In addition, potential adverse effects of interventions in terms of increasing inequalities should be considered in the EConDA models.

It was noted that policy makers are reluctant to make equity a priority when measuring cost-effectiveness. It was proposed that equity measures should be presented alongside the economic analysis but with caution in operationalising equity measures. Care needs to be taken to make the results of this analysis comparable across the range of interventions. It is beyond the scope of EConDA to include measures of equity or measurement of access to healthcare, however an important idea for future work (EConDA II). Inequalities however will be modelled and a scenario that shows how interventions can potentially widen or reduce health inequalities can be run.

The trade-off between reducing inequalities and increasing average health status should be considered (www.bmj.com/content/323/7316/762).

- **MODELLING**

A microsimulation model should be used in EConDA and analysis should differentiate between cohort and population-based analysis. This is a complex method and should be communicated in a way that is easily understood and with transparency. The same mathematical method will underpin each country model, however, the inputs will all be country-specific where possible.

- **QUALITY OF LIFE**

Measures of quality of life (QoL) should be included in the analysis, at least for some diseases such as musculoskeletal disease, where there may be no change in life years gained but changes in QoL with an effective intervention. EConDA can include QALYs.

There was discussion about which CE threshold to use. The WHO 3 x GDP option was discussed, but no consensus was reached on this. Fortunately, if you have the ICER (incremental cost-

effectiveness ratio) from the CEA and have chosen this threshold, you'll be able to calculate the INMB (incremental net monetary benefit) from the CBA quite easily. Another option mentioned was to calculate the ICER and present the INMB for several different threshold values.

- **CE THRESHOLD**

Cut-offs vary across countries meaning interventions may be cost-effective in one country but not in another. It was suggested that CE cut-offs should be reviewed. EConDA will use country-specific cut-offs when measuring CE. Economics guidelines for each country should be consulted as a starting point

Use of the WHO 3 x GDP option was discussed, but no consensus was reached on this. Fortunately, if you have the ICER (incremental cost-effectiveness ratio) from the CEA and have chosen this threshold, you'll be able to calculate the INMB (incremental net monetary benefit) from the CBA quite easily. Another option mentioned was to calculate the ICER and present the INMB for several different threshold values.

- **DATA**

Availability of data was a major point of discussion as more epidemiological and cost data are required. In particular, data about direct and indirect costs of diseases and costs of interventions. Partners were encouraged to give suggestions for costs' data sources. It was suggested that use of DALYs alongside QALYs be used, using data from the Global burden of disease study.

- **SILO EFFECTS**

Silo effects in policy making were discussed. Well-being is an inter-sectorial phenomenon, therefore policy makers from a variety of sectors need to work together.

There was no consensus about the discounting rates, only that they should be country specific. The OECD suggested that costs and benefits should be discounted at the same rate. Therefore, it was agreed that the model can include both arbitrary and country-specific discounting rates.

- **COSTS**

It was generally agreed that the reported costs should be health sector costs, with indirect costs reported separately alongside these.

We need high level agreement on how best to measure indirect costs and one size fits all approach is difficult.

- **COUNTRY SPECIFICITY**

A variety of aspects of economic evaluation should be country specific:

- Discount rate
- Cost of intervention
- CE threshold
- Way of calculating societal perspective
- Currency used

- **OUTCOMES**

Many different outcomes could and should be reported in CE analysis (DALYs, QALYs) so that a broader conclusion can be made about the value of a particular intervention, beyond any costs avoided for the health care system.

These outcomes need to be clearly defined. The EConDA models are able to produce a variety of

Economic evaluation enables comparison across interventions so policy makers can adequately allocate resource

- **RECOMMENDATIONS FOR ECONDA**

A number of recommendations came out of the discussion that should be considered for the EConDA modelling:

- Use of a discreet event simulation model rather than a Markov model should be considered to underpin each country model, which is then populated with country-specific data.
- Use of pharmocoeconomic guidelines to compare between countries
- Use of a country specific discount rate, cost of intervention, CE threshold, utility measures, way of calculating societal perspective, currency used etc.
- Presentation of confidence intervals should accompany outputs if possible

The ways forward in the field of CE analysis

In concluding the meeting, we aimed to bring together the discussion points from the two days to reach some consensus over the best methods for measuring cost-effectiveness for chronic diseases. These questions and responses are as follows:

1. What CE outcomes should the EConDA project model?

   It was suggested that different ICERs should be used: life-years gained; DALYs; QALYs; number of cases, direct costs and indirect costs

2. How do we measure direct and indirect outcomes?

   a) In terms of direct costs, it was suggested that tariffs are used or actual cost if available. Expert opinions were acceptable as were average costs
   b) Elements to include in indirect costs include: absenteeism/presenteeism. It was suggested that a friction costs approach as opposed to the human capital approach be used.

3. How do we best measure a societal perspective?

   It was suggested that the level of "out of pocket" costs should be considered, informal costs, and benefits such as "feel good" should be captured, though data availability means this may not be feasible at this point. A societal perspective should be country specific and account for local circumstances.

4. Should there be a cut-off point for CE and if so what should this be?

   It was agreed that there should be no standardised cut-off points as comparing CE is problematic. However, it was agreed that comparing cost per QALY could be useful.

5. Can we agree on an appropriate discount rate?

   There was no agreement on an appropriate discount rate. These are country-specific

6. How do we best compare across countries?

It was suggested that the most appropriate comparison is cost per QALY. CE depends on parameters that differ widely from country to country.

7. What are the ways forward and new methods beyond what currently exists?

   New research (Fischer et al, The Appraisal of Public Health Interventions: an overview; J of Public Health) is exploring the use of decision theory in appraising public health interventions. This combines different types of evidence given that RCTs are not feasible in PH interventions, and interventions applied to large groups are often underpowered. This area of research should be followed and considered in addition to the quantitative evaluation of intervention modelled in EConDA

   a) All relevant outcomes can be modelled as long as there are available data, though data availability will be an issue, at least for the foreseeable future.

   b) Indirect costs if used must have as much detail as possible; they could include, for example, productivity costs and presenteeism.

   c) The context for each country should be taken into account.

   d) Judgement-free research: no cost-effectiveness cut-offs should be applied to evaluate interventions. It is possible to compare prices per QALY, but should not directly compare CE across studies.

   e) Call for being explicit in the methods and data used in building the models.

Appendices

Appendix 1. Meeting Agenda

Appendix 2. Literature review

Appendix 3. Qualitative paper