

Minutes of the MAFEIP Workshop on the MAFEIP tool Brussels, 21 Sept 2015

1. Introduction

Peter Wintlev-Jensen, DG CNECT, opened the workshop and welcomed all participants. This was followed by a brief introduction by Fabienne Abadie, JRC IPTS, about the JRC's role in the development of a monitoring and assessment framework for the European Innovation Partnership on Active and Healthy Ageing (EIP on AHA) through the MAFEIP project. She gave a snapshot of the work on process indicators aiming to monitor the EIP on AHA process and a status of the research on outcome indicators which resulted in the model and tool to be presented at this workshop.

2. Conceptual framework for MAFEIP

Presentation

Christian Boehler, JRC IPTS, introduced the conceptual framework developed by IPTS. He underlined the challenge of monitoring over 500 commitments with variability in all dimensions (within and across Action Groups and interventions) with the subsequent variation in indicators used to measure outcomes. The bottom-up nature of the EIP on AHA did not allow collecting new data which added to the challenge.

The approach taken by IPTS over the last two years in MAFEIP was to focus on a limited set of outcome indicators commonly used across commitments and Action Groups that would allow linking up to the Triple Win (Quality of Life, sustainability of health and care systems, and innovation and growth) and ultimately to Healthy Life Years. This was done step by step, first selecting indicators then building a model that is generic enough to capture a variety of outcomes and subsequently implementing it as a web tool.

As a result, the tool developed allows users to estimate cost-effectiveness even at an early stage within the life-cycle of a technology, which can be used to inform decisions on further development or investment and, ultimately, to support business case development for a new technology for instance. This is an added benefit of the tool for commitments, while at partnership level the EC is interested in using the tool to aggregate impact in terms of health and health & care expenditure on population level and assess how the EIP on AHA is doing as a whole.

He showed how the model and tool can help identify whether a new intervention is dominant or dominated by current care, and how to make decisions if interventions are beneficial but more costly, which in turn requires considering a willingness to pay threshold (WTP). The model allows defining a cost effectiveness ratio (ICER) which can be compared to the willingness to pay threshold in order to decide whether a new technology should be regarded as cost-effective compared to current care.

Christian further explained the difference between QALY and HLY, and the rationale for choosing QALYs as a common currency in MAFEIP.

Decision Analytic Modelling (DAM) was chosen as the best suited approach to combine information from different sources so as to estimate certain outcomes within the MAFEIP tool. DAM requires data on four dimensions: probabilities, utilities, resources and unit cost for resources. Based on this, IPTS developed a generic Markov model with three health states (baseline health, deteriorated health, death) which allows simulating the progression between health states until the end of life of a patient or patient cohort.

Finally web implementation for the tool was chosen as a trade-off between coverage and data quality as it enables users to input data directly while IPTS would not be able to collect data on a wide number of commitments.

Last but not least Christian underlined that the tool can be used to assess impact of a technology throughout its entire lifecycle, starting from early stage development.

Discussion

- The model is based on the assumption that costs keep constant, while costs will decrease over time. IPTS clarified that one could use the tool in an iterative assessment, and update results whenever new information becomes available. Indeed, users can always take other points in time to repeat the modelling exercise.
- The use of the term 'technology' was found to be confusing as technology only represents a small part of what interventions are about. IPTS clarified that this is health economics jargon and that a technology could be a new care pathway, a social innovation or a device etc.
- The question of transferability to another location was raised and how the tool could cope with the difference in contexts. IPTS clarified that the tool is like an empty shell which is currently partly populated with country-specific data. However users can replace that data with specific data when they have such data available. As to knock-out criteria only those involved in the intervention can decide and judge if the tool can be used or not in specific situations.
- The question of whether the tool can take account of a situation in which a patient starts off from deteriorated health and moves back to baseline health was raised. IPTS and Panaxea clarified that in this version of the tool this is not directly considered. However one could change the parameters so that the utility attached to the health states would account for this, i.e. using utility weights that make the baseline health state worse than the deteriorated health state.
- It was asked whether there are plans to include more than three health states. Suggestions for future development consider the inclusion of more states of deteriorated health.
- The use of a Gaussian distribution was criticised as not being realistic. IPTS and Panaxea clarified that the choice of distributions was based on particular characteristics, such as beta distributions for probabilities and HRQoL as they are bound between 0 and 1 or lognormal distributions for cost as this distribution is only defined for positive values and skewed to the right. The user cannot insert other distributions than these but the parameters for these distributions can be specified by the user.
- The question of who will be users of the tool was raised. It is targeted at developers of "technology" to help them make decisions, at policy makers who can also use the

tool to take an investor's perspective to decide on which technology to foster, and at commercial investors.

3. Case study I: Falls prediction

Presentation

Leandro Pecchia, University of Warwick, discussed how using the MAFEIP tool helped him assess the potential of a technology. He presented the context for the falls intervention that they have been developing. Falls are due to complex and dynamic interactions between different factors with strong dependencies which make predictions difficult. He highlighted a number of intrinsic and extrinsic risk factors for falls. There are many technologies available today such as pressure sensor, ambient sensors, wearable sensors etc. These technologies are costly, they often cause false positives, which makes it difficult to predict falls and devices are perceived as obtrusive so they decided to use an existing technology that would not cause too many false positive and would not be obtrusive.

Two studies on biomedical signals, conducted in the previous months by Leandro Pecchia, were used as a starting point. Those studies identified a significant association between HRV and falling. 200 patients were enrolled, people assessed at the baseline were monitored for 12 months. All the falls were recorded. A HRV reduction was associated with a greater risk of falling. Based on this a second study was designed investigating how HRV could have been used to predict certain imminent falls. This demonstrated a significant and robust association of HRV features with the magnitude of blood pressure drops due to standing, which is one of the cause of some indoor falls. This predictive model was filed as a patent application and the plan now is to develop a device that allows triggering an alarm and act before a fall happens.

Christian Boehler then demonstrated how the work conducted by Leandro Pecchia and his colleagues was used to test the model. He explained the various data inputs and sources of input data used to populate the model in this particular case. The resulting graphs showing outcomes indicate the values at which the intervention (i.e. in this case the device envisaged) would be cost-effective. He also showed the impact on QALY and cost savings that could be made.

Discussion

- How does the tool address comorbidity? It can be addressed by feeding parameters for sub-groups of population which allows comparing outcomes for different sub-groups.
- What is the role of the willingness to pay (WTP) in that type of analysis as the final price for a technology has an influence on the willingness to pay? In this context, WTP reflects societal willingness to pay for a unit of health gain, not that of the individual for a certain product. In many healthcare systems with Health Technology Assessment (HTA) it is a means to inform allocation decisions. A number of methodological guidance have been published e.g. in the UK a threshold of 20-30K GBP per QALY has been defined. It is a decision rule that can be applied strictly or used as guidance. However if one sets wrong values one could deprive certain patients of the care they need. If a value is set too high, this may lead to technologies with lower cost-effectiveness entering a healthcare system, and this in turn may have

an impact on other treatments that cannot be paid for any longer. The tool allows setting different thresholds and performing a threshold analysis.

- Is it necessary to run the system with different ages? It may make a huge difference if one chooses the 65 or 75+ population. Age is another form of sub-group in the MAFEIP tool. One could run the model for different age subgroups and compare outcomes.
- The terminology "technology" or "tool" was found to be confusing by some participants. There is a need to better assess cost-effectiveness of technology but the issue is to collect data for this, including on costs. There is a need for new financial models. The costs shown for the device in the falls case was felt to be not representative of the cost of care associated with the use of that device. However IPTS clarified that other costs were taken into account in the model. Further it was explained that Warwick University did not only take the costs of the device into account but developed five different scenarios, e.g in hospital settings, in nursing home setting etc. Health economics attempt to assess incremental costs and health outcomes. The MAFEIP tool helps to identify scenarios and to decide in which care setting to first develop the solution.
- The age of 65 used in this case was questioned as the acceptance of such a device by people in that age category seemed less likely than for much older people such as 85+. It was explained that by using different transition probabilities in different age cohorts this could be addressed. Obviously many different patient factors (including but not limited to adherence) impact on device effectiveness, so that this again becomes a question of relevant subgroup analysis. In the future, it is planned to allow more nuanced data entries (i.e. age dependant transition probabilities for all parameters), but currently one would have to assess each subgroup individually with the tool.

4. Tool demo I

Christian Boehler run a live demo of the tool that was populated with the above data on falls, which illustrated how the tool works in practice.

5. Tool demo II

Presentation and demo

Lotte Steuten first gave some background information on the benefits and rationale for carrying out sensitivity analysis. Several types of uncertainty are associated with modelling in health economics, and the interpretation of results depends on the level of confidence or uncertainty in various factors. She referred to variability, heterogeneity and parameter uncertainty. Variability relates to the random chance that identical patients will experience different outcomes or costs, which is reflected in the standard deviations associated with the mean value. Heterogeneity relates to differences among patients that can be explained by their characteristics. Subgroup analyses help deal with this. Finally parameter uncertainty relates to the precision in the parameter (input) estimates of a model (e.g. transition

probabilities, costs, utilities). Sensitivity analysis helps to determine which parameters are the key drivers of a model's results.

She then demonstrated by using the falls case study as an example in the tool how such an analysis can be carried out, and how by varying parameter values in the model by a certain amount the model results change. She also showed an example of probabilistic sensitivity analysis using the data from the same falls intervention.

Discussion

- Further clarification was sought on the meaning of the intersection between the curve and the threshold. It was clarified that, for that particular parameter this represents the minimum value at which the related technology would still be cost effective at a given WTP threshold.
- The simplicity of the model was questioned. IPTS was asked whether there would be more than three states in the future as this would increase the degree of precision of results. However adding complexity is not always good in modelling, it can be done for specific cases but to fulfil the objectives of MAFEIP it had to be kept simple. It was also clarified that the optional inclusion of additional health states is a plan for further development of the tool and that this would be discussed in more detail in the wrap-up session.
- Further if one wanted to fund a new innovation, one could do a preselection using the model and then do a more detailed modelling on the best options, using an iterative approach.
- Users are asked to input minimum and maximum values in the model which can be difficult. It could be more convenient to use a fuzzy-logic with categories instead (high/medium/low). IPTS highlighted that Markov models rely on numerical data inputs which represent the best available evidence and this is not a tool for eliciting individuals' beliefs. Obviously there are many other approaches, but one aim of the tool is to allow users to enhance their ability to do modelling and it was very important to base this work on methods that are very widely accepted in the related scientific domains.
- Who should input data and who will be the beneficiaries of the system? Christian explained that we could have developed a model which would have to be populated by IPTS so IPTS would have had to collect data. This would have allowed for a more sophisticated and context-specific model and more efforts to obtain the best available parameter inputs. However, the problem with this approach is that only one or two case studies would have been feasible within MAFEIP, and this would clearly be unrepresentative of the EIP on AHA with its more than 500 commitments.
- It was asked why the cost of devices which are only a marginal part of the costs of care processes was used and not overall costs. IPTS clarified that on a conceptual level, in economic evaluation, by convention one only looks at incremental impact. One only looks at the costs related to an intervention that are likely to change. In this case we looked at a technology in a specific care setting. This also relates to the question of whether a device or technology aims to complement or replace an existing care pathway. In general, however, there is a need to incorporate more information and the tool should be developed further to incorporate more nuanced cost data next to the various options it already offers.

6. Case study II: Telehealth intervention for frailty

Presentation and demo

Gimon de Graaf, Panaxea, showed a video on the PERSSILLA telehealth intervention for frailty which provided information on the intervention and the context in which it is being implemented. Frailty is associated with higher mortality, higher health care consumption and lower quality of life and the intervention aims to screen for frailty and target pre-frail individuals to stop frailty progression. As a result of the PERSSILLA intervention, a reduction in mortality and health care consumption is expected, along with an improvement in health related quality of life

In this case two alive states were defined, baseline referred to pre-frailty and deteriorated health to frailty. The first screening data was used to feed into the MAFEIP tool. The aim was to assess the impact of the expected effects, check how sensitive these are, what the minimal required effectiveness level is for the intervention to be cost saving or cost-effective and identify the drivers of the cost-effectiveness. When new data becomes available in October 2015 the model will be run again and results compared to the prediction.

Discussion

- Clarification was asked on the scope of implementation. The intervention is implemented at local level in cooperation with the municipality. This reflects the recent changes in the Netherlands where municipalities have been granted responsibility for long-term care.
- Again the question of how the costs of the intervention were handled was raised. The cost of €19 per person per year reflected the cost of the service model once upfront investment had been made.

7. Wrap-up on the tool and general discussion

Presentation

Christian Boehler went through potential areas for further research and improvements to the tool.

So far the discussion had been about how the complexity of the tool could be increased to reflect clinical reality. This represents one point of view and one way to develop the tool further. Taking a policy perspective, the question is rather how much can processes be simplified while using reliable and robust data. The two perspectives should be reconciled.

Beyond improvement to the tool, what is missing more generally is further advancement in economic evaluation methods in the field of active and healthy ageing.

Discussion

- The question of how this work will be taken forward was raised, of who will use the tool and whether a new action area in the EIP on AHA should be created to deal with this.
- Arnaud Senn, DG CNECT, stressed the need to get more input, as there are different needs, and the MAFEIP tool has been designed to take care of different contexts and priorities. He urged participants to send comments to the EC, based on the workshop presentations, including on the kind of initiative that would benefit from being

monitored by MAFEIP. The feedback should include a clear description of the institution's needs, the working environment, and the objectives. Arnaud also underlined that the tool is far from representing the end of MAFEIP, but we now need to go further and for this input is needed. Arnaud will send a message to all participants to collect such feedback.

- To disseminate the tool one also needs to profile the stakeholders targeted. While refining and making the MAFEIP tool more user friendly is fine, another line of improvement is about how to get data and organise the data collection process. An area for improvement of MAFEIP is to work on existing methodology for gathering data.
- For the EC tools like MAFEIP or MAST only make sense if the data collection is right. This has to be considered in cooperation with existing attempts to improve data collection and methodologies.
- In terms of further deployment of the tool, there should be flexibility in data input. A user manual would be important as some users may find it difficult to use the tool. Further material may be needed but there are mouse-over texts and links to additional sources of information and references built into the tool, which already provide some guidance, including on methods.
- Dissemination is not the problem, it is education that is important. Users of the tool who are not most familiar with health economics learn a lot from using it.
- The tool helps facilitate the diffusion of health economics in an area where it has not been used much before. In pharmacoeconomics, it is very well established and decisions on allocation of resources are based on the methods presented in the workshop. The EIP on AHA and MAFEIP provide a platform to facilitate these methods and bridge the existing gap.
- IPTS is currently making final tests of the tool in the EC IT environment. Once this is completed within a 2-4 week timeframe the link to the tool will be shared with EIP on AHA stakeholders. Access to the tool is provided through ECAS.

8. Closing

IPTS and DG CNECT thanked the participants for all their valuable contributions and closed the workshop.