A Platform for Targeting Cost-Utility Analyses to Specific Populations

Elisa Salvi(✉), Enea Parimbelli, Gladys Emalieu, Silvana Quaglini, and Lucia Sacchi(✉)

Department of Electrical, Computer and Biomedical Engineering, University of Pavia, Pavia, Italy
lucia.sacchi@unipv.it

Abstract. Quality-adjusted life years (QALYs) are a popular measure employed in cost-utility analysis (CUA) for informing decisions about competing healthcare programs applicable to a target population.

CUA is often performed using decision trees (DTs), i.e. probabilistic models that allow calculating the outcome related to different decision options (e.g., two different therapeutic strategies) considering all their expected effects. DTs may in fact include a measure of the quality of life, namely a utility coefficient (UC), for every health state patients might experience as a result of the healthcare interventions. Eliciting reliable UCs from patients poses several challenges, and it is not a common procedure in clinical practice.

We recently developed UceWeb, a tool that supports users in that elicitation process. In this paper we describe the public repository where UceWeb collects the elicited UCs, and how this repository can be exploited by researchers interested in performing DT-based CUAs on a specific population. To this aim, we also describe the UceWeb integration with a commercial software for DTs management, which allows to automatically run the models quantified with the mean value of the target population UCs.

Keywords: Patient preferences · Utility coefficients · Cost-utility analysis · Decision trees

1 Introduction

Quality-adjusted life years (QALYs) are a popular measure for valuing the outcome of healthcare interventions in terms of resulting patients' expected life and perceived quality of life (QOL) [1]. To compute QALYs for a specific patient, his/her expected life is split in time intervals, each one (t_i) presumably spent experiencing a specific health condition. For each condition, a utility coefficient (UC) u_i can be elicited to measure the QOL perceived by the patient in relation to such condition. QALYs are then computed according to the following formula: $\sum_i ti * ui$.

Obtaining reliable UCs is fundamental, since the elicited values may be used in clinical practice for shared decision making (SDM) procedures guiding the personalization of care for a specific patient. Besides the use for the individual, UCs can be exploited at a population level, for cost-utility analysis (CUA). In the health economics

A. ten Teije et al. (Eds.): AIME 2017, LNAI 10259, pp. 361–365, 2017.
DOI: 10.1007/978-3-319-59758-4_44

context, CUAs are evaluations that compare alternative healthcare programs in terms of "cost per gained QALY" in the target population [2]. Eliciting UCs from a patient is not straightforward, and it is not a common procedure in clinical practice. Usually, UC values for specific diseases are collected during clinical studies, using either paper-based elicitation instruments or questionnaires designed for the specific circumstances [3]. Elicitation procedures usually require the presence of trained interviewers, to properly complete the process and obtain reliable UC values from the examined patient. CUA is often performed using decision trees (DTs), probabilistic models that allow calculating the outcome related to multiple decision options (e.g., two alternative therapeutic strategies, or whether or not to apply a preventive healthcare measure to a specific population) considering all their expected effects [4]. DTs may include among their parameters one UC for every health state patients might experience as a result of the compared healthcare interventions. Since collecting UCs is challenging and time-consuming, CUAs usually rely on UCs that are already available in the literature. Unfortunately, it is not straightforward to re-use such values, since they might have been elicited from a population that consistently differs from the one defined for the CUA. Using a sub-optimal set of UCs in CUA would introduce a bias effect in the analysis, and may consequently lead to sub-optimal decisions.

Few tools are available for eliciting UCs, and the majority of them has a limited range of application. For example, some of the tools described in the literature are embedded in disease-specific instruments designed to support targeted SDM processes [5], and are not exploitable for different decision analysis problems. On the other hand, general purpose elicitation tools are usually not well integrated with functionalities that allow the actual exploitation of the obtained values in either SDM processes or CUAs [6–10].

To support elicitation processes, we had already developed UceWeb [11], a web-application to elicit patient-specific UCs through three state of the art methods (time trade-off, standard gamble, and rating scale), and to collect them in a public repository for further use. In this paper we present a new functionality of UceWeb that has been recently implemented for supporting users in a more comprehensive decision analysis workflow. The new functionality allows the re-use of the UC values collected through our tool for targeting DT-based CUAs to a population of interest.

2 Methods

Researchers who perform DT-based CUAs on specific populations have two needs. First, to compute QALYs, they need to elicit a population-specific UC, and its confidence interval, for every health condition included in the model. Second, they need to effectively run the DT model quantified with such UCs.

For the first need, they could exploit the UceWeb public database where UC values are collected. In this repository, each UC is stored along with additional information on the related elicitation procedure, including the elicitation date, the elicitation method, the SNOMED [12] code identifying the health condition, and an anonymous identifier for the patient who has elicited the coefficient. For each patient, UceWeb collects a profile gathering relevant anonymous information such as age, sex, ethnicity, education, marital status, parental status, and occupation. Since patient profiles may be updated over time, the repository keeps track of any occurred modification. Thanks to the described framework, it is possible to query the UCs repository for retrieving the coefficients elicited from patients who fit a user-defined population (e.g. caucasian female aged from 35 to 55, employed in executive professions and having children). For targeting a DT-based CUA to such population, it is possible to use the mean value of the retrieved UCs as a population-specific parameter, which is in turn used to quantify the DT model.

To support users in employing the obtained population-specific UCs in DTs, we integrated UceWeb with TreeAge Pro [13], a widely used software for the formalization and analysis of DTs. The integration with the TreeAge Pro Suite was possible thanks to the TreeAge Pro Object Interface, which enables to open, update, and analyze DTs using programming languages (in our case, Java) and, consequently, to embed these functionalities in custom applications. To the best of our knowledge, this is the first attempt to integrate TreeAge Pro into a web-based application. To achieve this goal, we had to explore the Object Interface resources to assess which TreeAge Pro objects (e.g. variables and properties of the DT model) could be successfully managed via programming language. Thanks to the obtained integration, users are now able to run DTs formalized with TreeAge Pro and collected in a dedicated repository, directly from the UceWeb interface. The DTs repository currently contains two models, specifically formalized to address two decision problems: the optimization of the antiarrhythmic therapy for patients affected by atrial fibrillation (AF)("AAT model"), and the optimization of the anticoagulant treatment to prevent thromboembolic complications in the same patients ("OAT model") [14].

3 Results

This section describes the UceWeb workflow allowing users to perform DT-based population-specific CUAs.

First, the user can select the model from the ones available in the DTs repository. Then, thanks to the described integration with TreeAge Pro, UceWeb provides the user with the list of the health states considered in the selected model, whose UCs are required to quantify and run the DT. As an example, Fig. 1 illustrates the response returned by UceWeb when the user selects the "OAT" model. For each health condition, the user can decide to use the default UC values set in the model by the DT author ("Use default" buttons in Fig. 1). As an alternative, the user can decide to exploit the UceWeb UCs repository for "eliciting" population-specific UCs ("Elicit" buttons in Fig. 1). In this case, he/she is asked to fill in a dedicated form for defining the characteristics of the population to consider (e.g. age range, sex, type of occupation, ethnicity). On the basis of those parameters, UceWeb retrieves from the repository all the UCs elicited from patients who fit the specified population. It then computes the mean value, and presents it as the population-specific UC assessment required for the DT quantification. In the form, the user can also define a time frame of interest for the desired QOL assessment, limiting the retrieval to UCs elicited during a selected time period (e.g. consider only UCs elicited from January 2010).

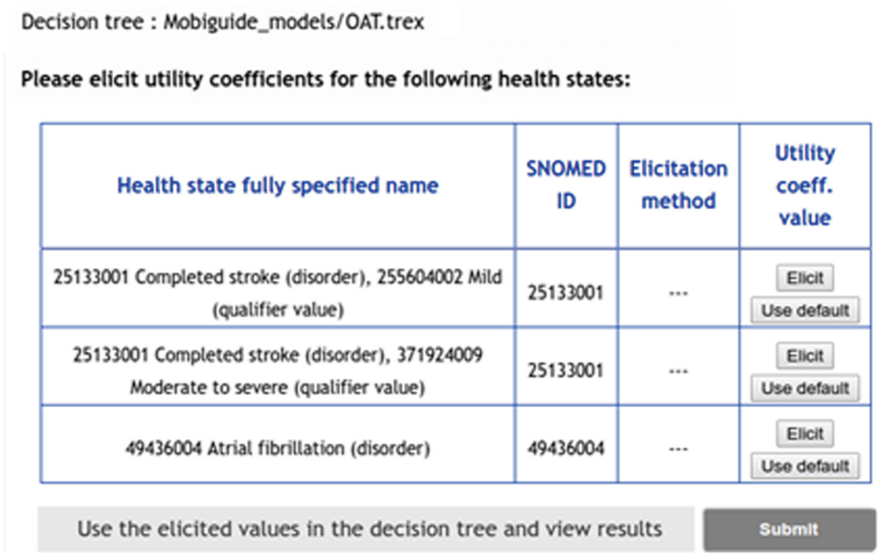

Fig. 1. List of the UCs values required for quantifying the "OAT" model.

When all the coefficients required for the selected model have been collected, UceWeb exploits the TreeAge Pro Object Interface for setting the obtained values into the selected DT, and to run the model. In this way, the analysis will be targeted according to the population assessed through our tool. Finally, for each decision option the results of the analysis in terms of QALYs and costs are presented to the user through the UceWeb interface.

4 Discussion and Conclusion

The aim of the described work is to provide an innovative tool for empowering researchers engaged in DT-based healthcare decision making processes. The proposed framework for bridging the gap between UCs elicitation and UCs exploitation in DT analyses has multiple advantages. First, it promotes the sharing and re-use of available UCs assessments, facilitating CUAs targeted to specific populations. Second, thanks to the ability to run any DT model formalized with TreeAge Pro, the UceWeb tool is domain-independent and thus exploitable in a wide range of applications. On the other hand, it allows users to perform disease-specific analyses once a specific model is selected from the DT repository.

Further steps are necessary to fully exploit UceWeb potentiality. First, we must promote the use of UceWeb in order to enrich its UC repository. In addition, we aim to expand the DT repository with models that are already described in the literature. Future work will also be focused on implementing a functionality to allow users to add their own models to the DTs repository. Since in healthcare decision making TreeAge Pro is widely used for DTs formalization, we believe that a significant number of researchers interested in CUAs have already used this software to formalize their own models. With a minor modification (i.e. the insertion of structured comments into the formalized DT), these models could be easily added into our DTs repository. This possibility should encourage researchers to use UceWeb as a collaborative platform for collecting DT models, increasing the value of the efforts dedicated to modeling disease-specific decision problems. Finally, we will define a validation procedure for assessing the usefulness of the described framework in practice.

References

1. Kind, P., Lafata, J.E., Matuszewski, K., Raisch, D.: The use of QALYs in clinical and patient decision-making: issues and prospects. Value Health. **12**(Suppl. 1), S27–S30 (2009)
2. Gold, M.R.: Cost-Effectiveness in Health and Medicine. Oxford University Press, Oxford (1996)
3. Dijkers, M.P.J.M.: Quality of life of individuals with spinal cord injury: a review of conceptualization, measurement, and research findings. J. Rehabil. Res. Dev. **42**, 87 (2004)
4. Weinstein, M.C., Fineberg, H.V.: Clinical Decision Analysis. Saundersm, Philadelphia (1980)
5. Thomson, R., Robinson, A., Greenaway, J., Lowe, P.: Development and description of a decision analysis based decision support tool for stroke prevention in atrial fibrillation. Qual. Saf. Health Care **11**, 25–31 (2002)
6. Bansod, A., Skoczen, S., Lenert, L.A: IMPACT4: a framework for rapid, modular construction of web-based patient decision support systems and preference measurement tools. In: AMIA Annual Symposium Proceedings, p. 782. American Medical Informatics Association (2003)
7. Sims, T.L., Garber, A.M., Miller, D.E., Mahlow, P.T., Bravata, D.M., Goldstein, M.K.: Multimedia quality of life assessment: advances with FLAIR. In: AMIA Annual Symposium Proceedings, pp. 694–698 (2005)
8. PROSPEQT: A New Program for Computer-Assisted Utility Elicitations. https://smdm.confex.com/smdm/2004ga/techprogram/P1523.HTM
9. Goldstein, M.K., Miller, D.E., Davies, S., Garber, A.M.: Quality of life assessment software for computer-inexperienced older adults: multimedia utility elicitation for activities of daily living. In: Proceedings of the AMIA Symposium, p. 295. American Medical Informatics Association (2002)
10. Sumner, W., Nease, R., Littenberg, B.: U-titer: a utility assessment tool. In: Proceedings Annual Symposium Computer Application in Medical Care, pp. 701–705 (1991)
11. Parimbelli, E., Sacchi, L., Rubrichi, S., Mazzanti, A., Quaglini, S.: UceWeb: a web-based collaborative tool for collecting and sharing quality of life data. Methods Inf. Med. **53**, 156–163 (2014)
12. SNOMED CT. https://www.nlm.nih.gov/healthit/snomedct/index.html
13. TreeAge Pro. https://www.treeage.com/
14. Rognoni, C., Marchetti, M., Quaglini, S., Liberato, N.L.: Apixaban, dabigatran, and rivaroxaban versus warfarin for stroke prevention in non-valvular atrial fibrillation: a cost-effectiveness analysis. Clin. Drug Investig. **34**, 9–17 (2014)