

Systematic evaluation, replication and validation of structural health economic modelling approaches

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SUMMARY



Summary

This dissertation studies the systematic evaluation, replication and validation of structural health economic modelling approaches in the field of obesity.

In particular it evaluates, replicates and validates the current structural modelling landscape in obesity, with an emphasis on commonly applied obesity-associated event simulation approaches. This research aims to increase trust and confidence in the selection and interpretation of results related to a specific methodological approach used as basis for decision analytic models in obesity. The research presented in this document is mainly informed by the content of five connected scientific publications (chapters 2-6).

In chapter 2, a systematic review on health economic obesity models is reported, which identified a total of 87 scientific articles. These 87 articles reported 69 unique modelling approaches, hence a huge diversity of obesity modelling approaches have been identified. This makes it difficult to compare and comprehend the model outcomes, as the structural and methodological differences could also have a major impact on the modelling results.

Chapter 3 focuses especially on the (diverse) **clinical event simulation approaches and the (lack of) external validation** in the health economic obesity models identified in chapter 2. This research found that one key limitation of these models is the lack of published external validation results. This is in spite of the valuable information provided by such methods on the predictiveness, and hence on the quality, of their event simulation approaches. Only ten model-based health economic assessments in obesity (14%; 10 of 72) performed an external validation and the predictiveness of the event simulation was investigated in a cohort of obese subjects in only one study. Future work on quality assessment of key structural approaches (expert panel) and on the comparison of most common event simulation approaches (cross validation & external validation) is required in order to guide future modelling in the field of obesity. This is presented in chapters 4, 5 and 6. Furthermore, the wide range of modelling approaches (identified in chapter 2) suggested the need to develop best practice recommendations for model-based health economic assessments in obesity.

Accordingly, **chapter 4**, reports on the methodology and results of an **expert panel rating on key structural approaches** used in the identified health economic obesity models, which were transformed into **(best practice) expert recommendations**. Focusing on the key structural aspects outlined in the Philips checklist, this research presents main findings relevant to obesity models that have been identified (systematic literature search), rated (expert interviews) and

discussed (expert panel). While the expert panel acknowledged the challenges in achieving consensus, several recommendations for key structural approaches for a health economic obesity model were developed.

Chapter 5, based on the systematic review and the expert panel recommendations (chapters 2 to 4), focuses on the selection and **replication of high-quality health economic obesity models** and on the **assessment of reproduction success**. This study confirms the feasibility of replicating complex obesity models, although some challenges were identified. Small changes to existing reporting criteria have the potential to increase the transparency of model reporting, and may increase the reproduction success of health economic modelling results, which may subsequently increase the transparency and acceptance of health economic modelling studies.

In **chapter 6**, the **influence of the (different) structural modelling approaches on the clinical event simulation and the health economics outcomes** is further investigated, and hence targets the research needs identified in the previous chapters. This research identifies that in a severely obese population, the structure of a health economic model matters if clinical events are to be predicted most accurately. However, if the purpose of a health economic model is purely the incremental cost-effectiveness ratio, this study suggests that the structure does not matter as much, as health economic results are fairly comparable. Further similar studies in other obese populations and in other disease areas would be needed to confirm the findings.

Finally, in **chapter 7** the main objectives and main results of the thesis findings are summarized and discussed in relation to the broader research context. In this chapter the main contributions of the thesis to the health economic research and to scientific debate are reported. Furthermore, the methodological challenges and considerations are discussed, and implications and recommendations for future research are provided.

This chapter highlighted that our research was able to identify some important aspects related to the health economic modelling methodology in general, and key aspects specifically related to the context of obesity. Our research could form a basis for evaluating the strength and weaknesses of different structural event simulation approaches, but also identified valuable future areas of research to further enhance trust and confidence in health economic modelling, especially in the context of obesity.