

Guidelines Take-home exam HEVAL5130 – Fall 2023

General requirements

A take-home exam gives the student an opportunity to show his/her analytical skills, and ability to synthesize information and marshal facts for a clearly articulated argument. Furthermore, a take-home exam tests if the student knows where and how to look up information and what to do with it. A take-home exam requires that references to literature and materials used in the text are correct and consistent.

Assessment guidelines

The report should be no more than 2500 words (not including tables, figures and reference list)

The grading will be based on two main categories (40%/60% valuation split):

1. Structure (understand what should be included in each section) **(40%)**
 - Follow the recommended structure
 - Content within each section was correct (For example, introduction did not include methods, results did not methods or theory, discussion did not present new results)
 - Comprehensive tables and figures with legends that include necessary information
2. Content and interpretation (show understanding of the theory, application, and interpretation of methods and findings) **(60%)**

The take-home exam will be assessed on the following guidelines:

- The take-home exam addresses the problem stated in a clear and consistence manner. Choices and delimitations made are clarified and substantiated, and the method chosen is presented, justified, and discussed.
- The application of methods meets scientific criteria (is methodologically correct).
- Discussion and analysis: Relevant empirical evidence is presented and critical discussed against the theoretical framework.
- The take-home exam should include reference to relevant peer-reviewed literature.
- Format: the take-home exam fulfill format requirements, meets the academic standards with regard to style, structure and deepening of the subject.

What determines the grade?

- Structure and length of the take-home exam must meet the formal requirements;
- The application of methods must be methodologically sound;
- Concepts, theories and empirical knowledge are presented correctly, and used in the discussion of the stated problem;
- The student must be capable of arguing in a logical and clear manner;
- There must be originality and critical reflection: the student must be able to indicate what he or she has learned or what he or she would do differently in a next study (note: this is not a matter of personal experiences but of academic insights);
- The student uses an approved reference style, without any errors;

- Layout of exam in academic style (appropriated titled figures and tables, and use of complete sentences and paragraphs);
- Result tables should not be copied directly from output files from STATA, R, or other statistical programs.

Special requirements and evaluation criteria:

All reports **MUST** include the following sections (sub-sections may vary by exam topic):

1. Objective
 - Topic 1.1: The objective needs to capture both evaluation of the cost-effectiveness of treatment A (CEA) by stratified/sub-group and also value of LUC.
 - Topic 1.2: The objective needs to capture both evaluation of the cost-effectiveness of treatment A (CEA) as well assessment of the decision uncertainty and potential need for further research (VOI).
2. Theory (presentation of the theory relevant for the chosen topic; peer-reviewed papers and/or book citations required)
 - Methods should not be presented here (no sentences such as, “in this analysis I assumed...”).
 - All methods/approaches used in the analysis should be explained in this section from a theoretical perspective.
 - Topics 1.1 and 1.2: Theory on economic evaluation (and ICERs and Net Monetary Benefits) and decision modeling to estimate outcomes should also be included, including validation of decision-analytic models, and uncertainty in decision modeling (i.e., probabilistic analysis and preferably the other types of uncertainty should be mentioned). Both topics also require a presentation of survival- and hazard functions, censoring, time-dependency, non-parametric versus parametric. The student should include the importance of smoothed hazard plots, visual inspection and goodness of fit (AIC and BIC).
 - Topic 1.1: Additional survival theory on testing for proportional hazard assumption, as well as on stratified analysis/heterogeneity, LUC and incremental net benefits from stratification (citing Coyle et al).
 - Topic 1.2: Theory on value of information, define and explain the theory (EVPI, popEVPI and EVPPI) and how these values are theoretical maximum values of collecting additional research and why you might need EVSI. Distinguish between gold standard method to estimate EVPPI and the approximation methods. Theory on how to scale to population level, and design of trials (e.g., grouping parameters for EVPPI). Mention why probabilistic draws often need to utilize Cholesky decomposition. Finally, some theory on structural uncertainty analysis.
3. Methods (depends on the topic)
 - Theory and results should not be in this section (equations should generally be in the theory section). Definitions of concepts should be in theory, not generally in methods.
 - In general, the text should NOT reflect the mechanistic detailed tasks that were performed, e.g., “I copied and pasted worksheet x”, but rather described methodological assumptions and approach in a “report-based” methods format, e.g., “we adapted the baseline model to reflect x, y, and z”; or, “we assumed that there were x number of patients over a y year period.”

- Topics 1.1 and 1.2: In this section, it is required that the candidate describes the decision-analytic model including health states and important assumptions (e.g., assumptions associated with transitions between health states, strategies considered, model outcomes), and presents the data used for the analysis, defines the variables used in the analysis. All other assumptions made in the analysis must be specified, including time horizon, discount rate and perspective (analysis transparency). Could include impact inventory in appendix. Describe glioblastoma cancer dataset, and the variables used in the analysis. Analysis needed to convert monthly health outcomes from the model, i.e., monthly quality of life into years in order to estimate quality-adjusted life years; otherwise, the “ICER” for a yearly WTP will not be in the same units. Needs to describe approach to decision-analytic modeling and analysis validation. Methods should include a presentation of estimating preferred parametric distribution, e.g., using the smoothed plot of overall hazard over time to choose between distributional forms that captures the hazards rates over time, AIC/BIC, visual inspection.
 - Topic 1.1: Can test for PHs, and AIC/BIC (to determine inclusion of covariates). Model adapted, included for treatment effect (RRs), costs and utilities for the comorbidity groups. Make sure treatment effect only applied for the first 12 months. LUC criteria assumptions (patient population proportion, number of patients, WTP thresholds evaluated). Explain the motivation of EVPPI as a way to evaluate decision uncertainty (could touch on research priorities, but for 1.1, the motivation was more about using EVPPI as a type of sensitivity analysis to assess parameter contribution)
 - Topic 1.2: Present input parameters and the assumed probability distributions (report transparency). Provide all assumptions made in VOI analysis, including data used to inform input parameters, estimation of the population to benefit from research, discount rate, WTP, proposed research studies and assumed expected costs of research studies. The grouping of input parameters to perform EVPPI should be presented and justified in this section, e.g., Weibull parameters can be collected in a new observation trial, while relative treatment effect would often require an RCT. Preferably a table outlining these assumptions. All parameters should be included in a group and explored (including costs, which had high EVPPI in both Center B only analysis and the Center A/B analysis).
4. Results (assessment includes review of calculations/coding in Excel file/R code)
- Both topics: Clear presentation of the findings in a logical order, and according to questions asked in the home-exam (if statistical analysis/methods were defined in this section, it is a negative with regard to structure, but not to content).
 - Topic 1.1: Needed to adjust incident death calculations (rather than leaving the default constant transition probability (e.g., ‘Model_Treatment_A’)). Importantly, time-dependent survival was affecting both treatment groups; therefore, models needed to adjust both Treatment A and B for time-dependent stage- and age-specific death probabilities (not only Treatment B). Cumulative number of cancer deaths should be presented as a cumulative risk, or the report could have used the comorbidity-specific proportion of 1500 total glioblastoma individuals (but not 1500 in each group). Model should have correctly incorporated sub-group indicator variables, and presented the groups for which Treatment B was considered cost-effective for the varying WTP thresholds, as well as the weighted population ICER (to enable comparisons to sub-group

LUC). Shape and Scale should be included as recorded variables (and NOT the monthly transition probability itself).

- Topic 2: Presentation of the probabilistic results including: QALYs, costs, incremental QALYs, incremental costs, and the incremental cost-effectiveness ratio (ICER) (analysis transparency requires all presented). Interpretation of the results assuming that your WTP of GBP40,000 per QALY. Visualization of the CEAC and CEAF results and interpretation of these results. Explicit step-wise interpretation of VOI results, i.e., the population EVPI results, assuming that the expected research costs are between GBP 0.5-10 million depending on the research study design, and that your WTP threshold is GBP40,000 per QALY. Interpretation of the population EVPPI results with justified study designed (here costs were a major driver of EVPI). Visualization of all VOI results and/or presentation in form of a table. There should be no statements that further research is definitively worthwhile based on pEVPI or pEVPPI alone, only that it is potentially worthwhile / might be worthwhile (we cannot know for sure without EVSI).

5. Discussion

- Discuss the results, refer to other literature when relevant was a bonus, how general are the findings, etc. Limitations.
- Topic 1.1 could have discussed ethical implications of LUCs.
- Topic 1.2: Elaboration on whether the decision maker should make an immediate decision or collect additional evidence and explain the next steps, such as EVSI and ENBS (briefly that EVSI and ENBS would be needed to determine whether further research is worthwhile).

6. Conclusion

- Should be in line with the objective, defined in the introduction of the home exam
- Topic 1.1: Conclusions should comment on the cost-effectiveness of Treatment B compared with A (based on the weighted average ICERs), and how it differed between the sub-groups at the WTP.
- Topic 1.2: Conclusions should comment on the cost-effectiveness of Treatment B compared with A, the robustness of this conclusion across the scenarios, and, depending on the assumed cost of the research, whether a decision should be made today (because research costs would exceed EVPPI group value) or the next steps of VOI (i.e., EVSI and ENSB) should be evaluated.

7. References

- Consistently referenced and styled

8. Appendix

- Must include R/do file script (Topic 1) as an appendix, and completed Excel model (both topics) as the additional file.