Improved methods and actionable tools for enhancing HTA

An analytical framework outlining the determinants of HTA recommendations across settings based on primary data collection and primary analysis of secondary data

Executive summary

Work Package 7 - Deliverable 7.1 (Task 1)

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Background

In order to allocate scarce resources efficiently, health policy-makers have become gradually interested in developing and implementing methods of value-based pricing for the assessment of new medical technologies. The main aim of these tools is to allow policy-makers to perform informed decisions on whether health technologies should be reimbursed by assessing their value in relation to their opportunity costs. To this end, health technology assessment (HTA) has been implemented in a growing number of countries to support decision-makers with choices regarding the services and products to be made routinely available as part of national healthcare systems.

Nevertheless, HTA is not always implemented in a similar manner across settings. Studies have identified variations in the evaluation of evidence, agency structures, and guidelines for conducting HTA. Considering the multiplicity of factors that feed into the value of new medical technologies, there is a need for improving available tools and methods for assessing their value, as well as overcome the limitations of the current approaches.

Objectives

Within the European context, although health and social policy remain a responsibility of the Member States, an opportunity exists for improving the effectiveness and efficiency of health technology procurement by implementing cross-country collaboration in a range of activities, including Health Technology Assessment (HTA). This certainly applies to the field of methodological development, but also in that of empirical and applied study. In some cases (e.g. RCTs and other clinical studies) the results of a specific study can be almost directly applied to other settings, but in the case of economic evaluations, a number of aspects must be customized to country-specific circumstances in order to attain external validity. Recent years have also seen significant changes such that data on costs and health outcomes are now available from an increasing range of sources underscoring the need for better data integration and evidence synthesis, as well as the strengthening of data generation processes for economic evaluations and the improvement in methodological quality. Based on these challenges, the overall objectives of TASK 1 of work package 7 (WP7) are:

First, to create an analytical framework explaining the determinants of HTA coverage recommendations linking the quality of evidence, social value judgements and other considerations that may be relevant to decision-makers. Methodologically, task 1 selects a sample of approximately 200 drug-indication pairs that have undergone HTA during the 2009 – 2018 period across different

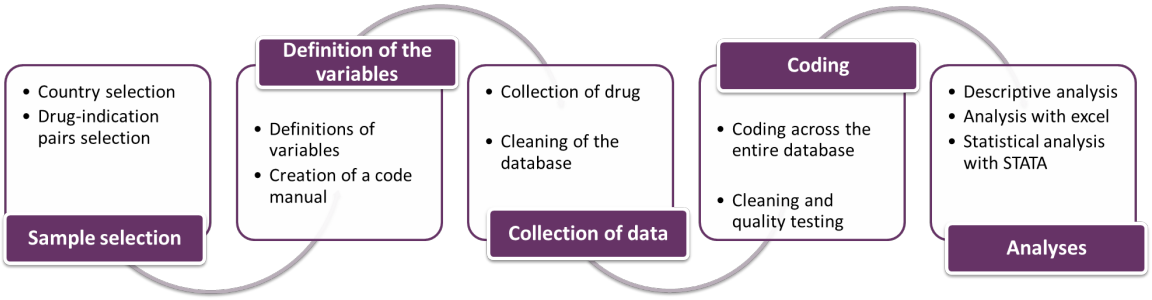
jurisdictions and for which HTA reports and recommendations are available. Country/HTA agency selection will be based on different models of HTA as they apply in different jurisdictions. Drug-indication pairs will be selected from across therapeutic classes and based on what HTA agencies have assessed/appraised during the above study period.

Second, based on the primary objective, a methodological framework will be created to inform the data collection process, which builds on previous attempts and in order to identify critical variables in the evidence submitted to HTA bodies (both clinical and economic, if the latter only where it is applicable), the interpretation of the evidence from HTA bodies (including the scientific and the social value judgements made) and seek to identify components of uncertainty as well as elicited and non-elicited other considerations that may have played a role in the assessment/appraisal process for each drug-indication pair. It is envisaged that several variables will be collected this way, which will then be included, coded and categorized in a spreadsheet in readiness for analysis. Econometric analysis will be used to identify the determinants of HTA outcomes, the role or importance of precise criteria that have been used in each setting to make coverage recommendations and will estimate the effect of different evaluation parameters. This process will feed into the creation of a value framework outlining the importance and relevance of different parameters of value across settings. In a final step, this evidence will be used to triangulate with HTA agencies to discuss and validate findings as well as collect individual feedback on the thinking and criteria applied in individual cases.

Methods

The collection and analysis of HTA data across countries is based on a multi-stage framework aiming to capture in a systematic way the key elements of HTA processes. Five steps were followed (Figure 1) in order to select the sample, and collect and analyze the data.

Figure 1 - Flow of data collection process



Country selection will be made in order to have a representative sample of the different approaches adopted in HTA (e.g. health service, societal), whilst at the same time ensuring data availability and comparability across settings. In order to select a representative sample of drug-indication pairs that have undergone HTA between 1st January 2009 – and 1st January 2018, across different jurisdictions and for which HTA reports and recommendations are available a two-step approach will be adopted: (1) Identification of all drug indication pairs assessed by selected HTA agencies over the study period; and (2) selection of representative sample of drug-indication pairs.

Step 2: In order to capture in detail key elements included in HTA reports, a comprehensive set of variables will be extracted from each HTA report and inserted into an excel spreadsheet. The construction of a composite set of variables will enable an understanding of the differences in evidence across HTA bodies and differences in the assessment of medicines across settings. This will allow the identification of key commonalties and differences both, in the evidence per se (and therefore at assessment level), and in their interpretation at appraisal level.

Steps 3 & 4: The variables will be organized according to 6 macro-level headings and coded accordingly: (a) general information, containing all the information regarding the key dates and outcomes of the HTA decision; (b) Clinical evidence, summarizing all the clinical evidence considered by the HTA body; (c) Economic evidence, capturing information on the economic models considered by the HTA body; (d) Risk sharing agreements, capturing all the (financial and non-financial) agreements between pharmaceutical companies and health care systems to mitigate risk; (e) Uncertainties around the clinical and economic evidence presented; and (f) Social Value Judgements (SVJs), interpreting key elements related to the impact of the treatment on the patient and the society (e.g. unmet need).

Step 5: Analysis will be both descriptive and quantitative (econometric), using linear, logistic and multinomial logistic regression analysis together with a number of additional tools that will apply to certain categorical variables, e.g. principal component analysis in the context of clinical & economic uncertainties and the social value judgements.

Analysis and outcomes

The sample will be analysed with different econometric modelling techniques in order to study the correlations between the different determinants of HTA and the recommendations outcomes. A number of peer review papers will be produced based on the data collected, which is aiming to inform the predictors of HTA outcomes. Two research articles are expected to be published during the course of 2022, as shown below:

(a) Algorithms and heuristics of health technology assessments: A post hoc analysis of factors associated with funding decisions for new drugs across eight OECD countries; and

(b) Health Technology Assessment and funding policies for Orphan Medicinal Products: Failing, Flawed or Fair?

These articles are included draft format in the full deliverable D7.1 and are currently being considered for publication in the peer review literature. As the review process is ongoing, the full D7.1 will be made available on the IMPACT-HTA website (www.impact-hta.eu) in January 2023, once the peer review process has been completed and the papers have been published.