POSTER
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CLINICAL VALIDATION OF A BUDGET IMPACT MODEL USING THE ESTABLISHED KEY OPINION LEADER VALIDATION FRAMEWORK (KOLVF)

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PURPLE SQUIRREL ECONOMICS

BACKGROUND

FACE VALIDITY

- Clinical validation also referred to as face validation, is a critical requirement for any health economic (HE) analysis, including models, assumptions, and interpretation which is considered as the first order validation (1).
- Face validity is defined as the act of evaluating whether HE analysis can clinically and medically represent the health problem it intends to address (2). It is subjective and mostly qualitative, so that clinical key opinion leaders (KOL)s in the problem area are consulted for face validation in HE studies (2–4).

GUIDELINES

- Guidelines suggest clinical validity should be considered in all economic evaluations, but specific timings for such a costly and time-consuming consultation have not been provided (1,3–6). The Canadian Agency for Drugs and Technologies in Health (CADTH) advises that the consultation should be done early and "iteratively" during the analysis process (4). Moreover, no specific guidance is available on how to ask HE questions to clinical experts who predominantly are not familiar with HE concepts.
- Previously, we established a practical framework for the clinical validation of HE models (KOLVF), which provides a novel time-oriented HE model validation framework (Table 1) (7). However, this framework has never been used in a HE project.

Table 1: Clinical validation process

	WHEN?	WHO SHOULD BE CONSULTED?	METHOD
FIRST CLINICAL VALIDATION	Early, at the start of the model conceptualization	Three or five clinical experts in the problem field and from different practice settings (should be blind to any possible results)	Modified Delphi panels or interview
SECOND CLINICAL VALIDATION	Early, consultation for model inputs Early, consultation for the structure of the finalized model.	Three or five clinical experts in the problem field and from different practice settings (should be blind to any possible results)	Modified Delphi panels
THIRD CLINICAL VALIDATION	Late, evaluation of consistency of the model's output with clinical expectation.	Three or five clinical experts in the problem field and from different practice settings	Modified Delphi panels or interview

OBJECTIVE

The current research objective was to generate a case study to demonstrate the usefulness of applying the KOLVF for the clinical validation of a budget impact model (BIM). We chose a new medical device developed to improve the management of osteoarthritis (OA) to present our findings.

METHODS

In concordance with KOLVF, three rounds of consultations were performed involving three clinical experts from different practice settings. (Blue boxes)

- The first occurred at the model conceptualization to gain insight on clinical situations, relevant target populations, treatment pathways, and feasible comparators.
- The second was conducted after initial input of real-world evidence and clinical trial data into the model, to solicit clinical advice on necessary model adjustments.
- The last consultation was performed after obtaining the results to detect if all the relevant questions were addressed transparently by the model.

RESULTS

- After providing the first structure of patient flow and treatment pathway by HE team and considering the clinical trial data, the severity of OA was defined by KOLs as Kellgren and Lawrence grade, where grade 2, 3, and 4 indicated mild, moderate and severe OA, respectively, through the first round of consultation. Moreover, the outcome of treatment was defined as the adherence to current treatment and the need for total knee arthroplasty (TKA).
- Through the second inquiry, the existing gaps for epidemiology and cost data were discussed and scientific answers provided.
- In the last face validation, the consistency of results with real world practice, and the transparency of results were evaluated.

(Red boxes)

The first structure of patient flow and treatment pathway provided by HE team by considering the clinical trial data

Inputs (epidemiology and costs) provided from literature review and patient-level data

Results of the budget impact analysis (BIM) provided

START OF MODEL CONCEPTUALIZATION

1

2 **FIRST CLINICAL VALIDATION**

Questions:

- What are the underlying clinical situation, disease stages, relevant target population, treatment pathway, feasible comparators (including standard of care), and background care?

3 **Revise the draft conceptual model**

PROVIDING INPUT DATA

4

1. Systematic literature review
2. Meta-analysis or NMA
3. Targeted literature review
4. Search official sources and data bank
5. Patient-level data

5 **SECOND CLINICAL VALIDATION**

Questions for the model's inputs:

- What are the proper perspective, time horizon, and geographical location (if applicable)?
- What is the availability of best/alternative data sources?
- How should data gaps be filled (e.g. unavailable health-related quality of life)?
- How should costs and resource use be calculated, which match clinical experience?

Questions for consultation for the structure of the finalized model:

- Does the model contain all clinically relevant aspects of the decision-making process, and is the model consistent with clinical practice?
- Is the objective of the model consistent with the research question?

6 **Revise the inputs and adjust the model based on available data**

FINALIZING THE RESULTS

7

1. Providing the results (costs, effectiveness)
2. Performing sensitivity analyses

8 **THIRD CLINICAL VALIDATION**

Questions:

- Are the results plausible compared with the clinical setting? Are health outcomes (QALYs, deaths) and economic outcomes (total costs, hospitalization) relevant?
- If there are any weaknesses in the model structure, inputs, and results, how well are the discrepancies addressed?
- Is transparency considered in all fields?

9 **Minor adjustment to address the final concerns.**

REPORT FINAL RESULTS

The OA severity index used in the model was challenged, and the KOLs helped to refine the segmentation of patients and consequently grade 2, 3, and 4 were defined as mild, moderate and severe OA, respectively. Moreover, the outcomes of treatment were redefined as the adherence to current treatment and the need for total knee arthroplasty.

Additional data gaps in epidemiology and costs were validated, and some assumptions were also confirmed or slightly modified by the KOLs.

The KOLs ensured the results were consistent with real-world practice.

DISCUSSION

- We previously suggested a practical framework for the clinical validation of HE models (KOLVF) which included three rounds of consultation with a KOL panel.
- KOLVF framework was used in the development of a BIM on the management of OA to examine its efficacy and to improve the BIM design.
- This new framework could help clinical KOLs to be prepared for each round of consultations to provide better support for HE teams. Moreover it was useful for HE teams to propose more relevant questions for clinical problems of their model.
- KOLVF is a bridge between clinical KOLs and HE teams to prepare for three rounds of targeted questions; model conceptualization, providing input data, and finalizing the results.

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CONCLUSION

The timing of clinical validation is key to maximize the value from the assistance provided by KOLs in the design of the conceptual framework and in filling data gaps. The designed KOLVF showed a great positive impact in the quality of clinical validation.