Economic Model and Methodologies Employed in Recent NICE Single Technology Appraisal Submissions

Dasari M[1], Cook JR[1], Dhankhar P[1]

[1] Complete HEOR Solutions, North Wales, PA, USA

Background

The National Institute for Health and Care Excellence (NICE) is a health technology assessment (HTA) agency, provides advice and national guidance to improve health and social care in United Kingdom (UK)

NICE technology appraisals guidance evaluates both cost and clinical effectiveness of health technologies to ensure that the intervention is effective and safe enough for use in the National Health Service (NHS)

- Technology appraisals can be of the following forms:
  - Single Technology Appraisal (STA): Assesses a single technology for a single indication
  - Fast Track Appraisal (FTA): Covers single technology for a single indication but with a shorter processing time window
  - Multiple Technology Appraisal (MTA): Covers more than one technology or a single technology for multiple indications
- The focus of this investigation was on STAs

Objectives

Primary Objective:

- To summarize the type and frequency of economic model designs used in recent NICE submissions

Secondary Objectives:

- To assess the trend of NICE submissions by various therapeutic areas
- To estimate the proportion of submissions by types of economic model and further assess time trends
- To describe reasons for NICE's rejection and/or partial recommendation of a drug/technology

Methods



1 REVIEW

- A review of all NICE STA submissions from June 01, 2014 and May 31, 2019 was performed

2 EXTRACTION

- Data were extracted on therapeutic class and various methodological parameters of the economic model (e.g. model type, time horizon, cycle length, utility measures, outcome variable)
- Furthermore, drivers/reasons for rejection and partial recommendation was collected and summarized

3 ANALYSIS

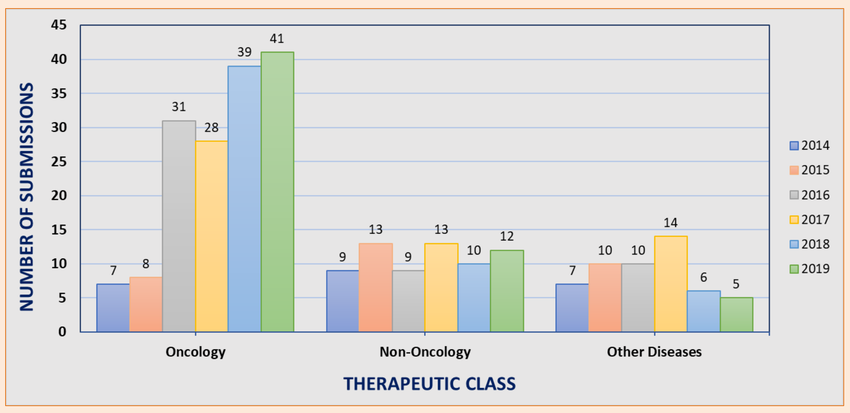
- Results were tabulated in an Excel spreadsheet and descriptive statistics were compiled
- Analyses by year, therapeutic class, and recommendation status were conducted

Results

I. Trends in NICE Submissions by Disease Area

- A total of 228 STAs were reviewed
- More than half of the STAs were submitted for oncology (55.7%)
  - Within oncology, the most frequent cancer type was NSCLC (16.5%) followed by leukemia (14.2%), breast cancer (10.2%) and melanoma (10.2%)
- Among non-cancer diseases, psoriasis (8.9%) had the most submissions followed by hepatitis C (6.9%), heart diseases (6.9%) and T2DM (5.9%)
- Remaining HTA submissions were grouped into "Other Disease" categories (20.2%)
  - Other diseases include ankylosing spondylitis, anaemia, Crohn's disease, depression, deafness, kidney disease, kidney or liver transplant, etc.
- **Figure 1** displays the frequency of STA submissions by disease area from 2014 to 2019

Figure 1: Number of STA Submissions to NICE by Disease Area and Year

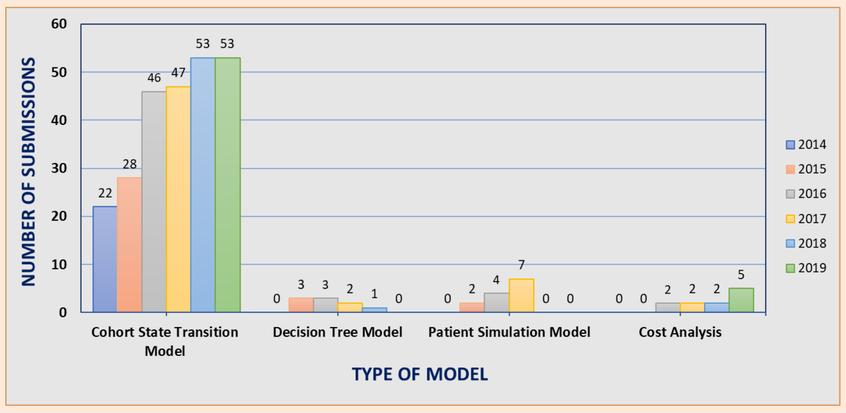


* For meaningful comparison, counts from June 2014 to December 2014 and from January 2019 to May 2019 were inflated for a full year; the actual number of submissions during the study period were as follows for 2014 and 2019: Oncology (4 & 17); Non-oncology (5 & 5); Other diseases (4 & 2)

II. Types of Economic Model Employed in NICE Submissions

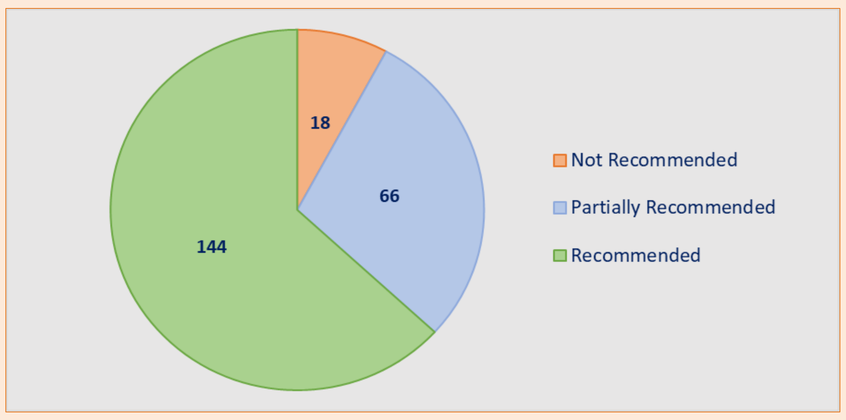
- Most of the submissions (91.7%) utilized a cohort state transition model (partition survival, Markov, or semi-Markov model)
- Less frequently used model designs were individual patient-level simulation (5.7%), decision tree (3.9%), and simple cost analysis (3.5%)
- A small number of submissions (4.4%) utilized multiple types of economic models
- **Figure 2** depicts year-wise frequency of different types of economic model used for NICE submission
- **Figure 3** show the outcomes of the 228 HTA submissions

Figure 2: Distribution of STA Submissions by Type of Model and Year



* For meaningful comparison, counts from June 2014 to December 2014 and from January 2019 to May 2019 were inflated for a full year; the actual number of submissions during the study period were as follows for 2014 and 2019: cohort state transition model (13 & 22); decision tree model (0 & 0); patient simulation model (0 & 0); cost analysis (0 & 2)

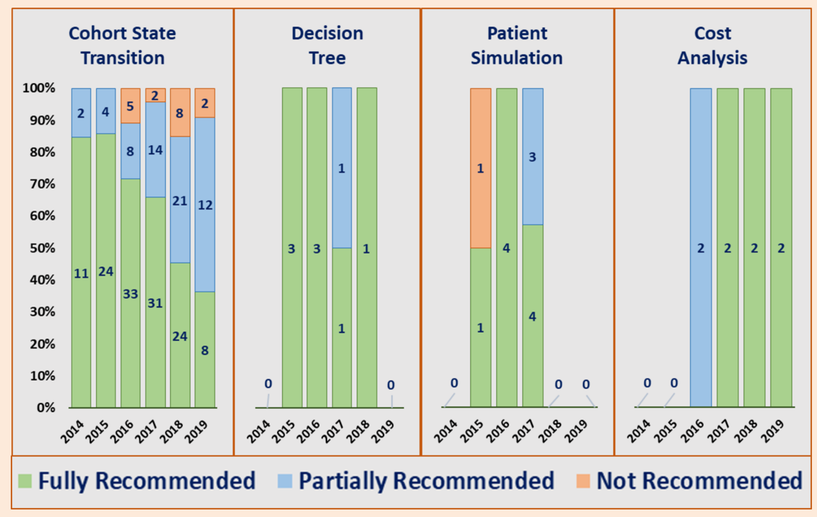
Figure 3: Distribution of Decisions by NICE for STA Submissions



III. NICE Recommendation by Type of Economic Model

- Most submissions utilized a cohort state transition model, with 62.7% fully recommended by NICE followed by partial recommendation (29.2%)
- Although less commonly used, 88.9% of the submissions based on a decision tree model were recommended by NICE
- Similarly, cost analysis is not a very popular technique for NICE submission but, when used, 100% of such models were either fully or partially recommended
- Among patient simulation models, 69.2% were fully recommended by NICE, with the rest either partially recommended (23.1%) or not recommended at all (7.7%)
- **Figure 4** depicts year-wise recommendation rates of models submitted to NICE by types of economic model
  - There appears to be a consistent decrease over time in the percentage of submissions to be recommended when the cohort health state model is used

Figure 4. Distribution of Decisions for STAs by Type of Model and Year



* Counts in 2014 and 2019 do not reflect the full year

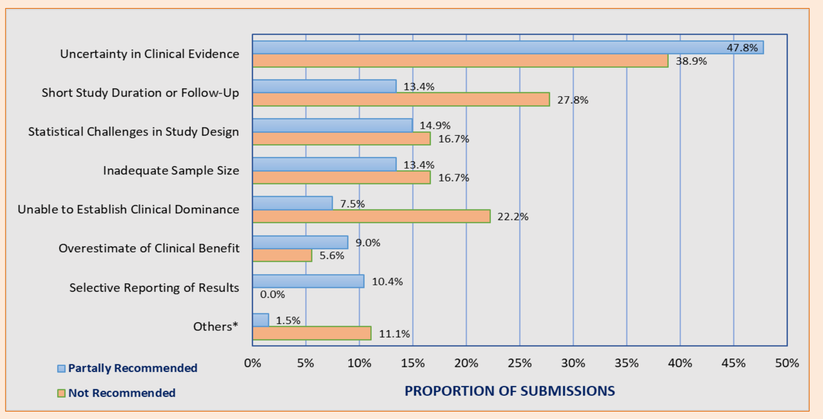
Additional Observations from the Study of Economic Evaluation Methodologies

- Cycle length varied substantially within a given indication (e.g., from 1 week to 4 weeks for non-small cell lung cancer)
- The most common instrument for collecting utilities in a clinical trial was the EQ-5D
- Other generic and disease-specific utility measures used included EORTC, DLQI, SF-6D, SF-36 and HUI-3

IV. Reason for Rejection or Partial Recommendation by NICE

- Economic Reasons (for rejection / partial recommendation): high cost and an ICER above the threshold
- Clinical Reasons:
  - The most common reason was uncertainty in the clinical evidence, leading to either partial recommendations (47.8%) or no recommendation (38.9%)
  - Clinical Trial Design Drivers: Inadequate sample size, short study follow-up, selective reporting of results, statistical challenges
  - Clinical Benefit Drivers: Overestimate of clinical benefit, unable to establish clinical dominance, lack of direct evidence from clinical trials
- Interestingly, none of the models were completely rejected due to lack of transparency/selective reporting of results

Figure 5. Clinical Reasons for NICE Rejection or Partial Recommendation



Conclusions

- The analysis provides context for trends in NICE STA decisions
- Cancer was the most common (and rapidly increasing) disease area for submissions to NICE since 2014
- Cohort state-transition models with cost-utility analyses were the most common design used in the NICE submissions
- More than half of the submissions resulted in a full recommendation; among the remaining submissions, NICE was more likely to provide a partial recommendation than to not recommend at all
- The likelihood of a full recommendation, however, declined over time in submission that utilized a cohort state transition model (esp. partition survival models); lack of a recommendation was often due to inadequate clinical evidence (insufficient follow-up and/or substantial uncertainty)
- Results of this review can serve as a guide for developing economic models used in NICE submissions

References

- National Institute for Health and Care Excellence (NICE), Technology appraisal guidance (URL: https://www.nice.org.uk/guidance/published?type=ta), Accessed on June 11, 2019

Poster presented at the ISPOR Annual European Congress, 04-06 November 2019, Copenhagen, Denmark