

Efficient and high-quality decision making: an exploration of disease-specific reference models at NICE

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Background

NICE develops guidance for the NHS in England, including Clinical Guidelines, HealthTech Evaluations (including medical devices, diagnostics and digital technologies) and Technology Appraisals (TAs) (including medicines). These are based on health economic modelling, which follows a set of rules known as the NICE reference case. The reference case has reduced inconsistency between models and the guidance they inform. However, some inconsistencies between economic models remain, and this will be more evident for some diseases than others.

It was hypothesised that disease-specific reference models¹ or disease-specific reference case extensions could help to further improve quality and reduce inconsistencies, therefore helping NICE to bring its existing guidance together in a useful and usable form.

This project aimed to inform the development of a position statement.²

What we did and why

- To assess the benefits, challenges and feasibility of using reference models, we
- Conducted a comprehensive literature review on the topic
 - Collated learning from previous NICE experiences (Box 2)
 - Surveyed other HTA organisations to explore their experiences
 - Settled on terminology that fitted the needs of NICE (Box 1)
 - Developed an internal position² to be applied across NICE’s guidance programmes (Box 4)
 - Outlined next steps (Box 3)

Benefits & challenges

- Enhanced Methodological Consistency**
- Disease-specific reference models can improve consistency across guidance products and reduce duplication of effort.
- Transparent Decision-Making**
- Reference models support transparent and predictable decisions, building stakeholder confidence and engagement.
- Resource Intensity Challenges**
- Developing and maintaining reference models requires significant resources, careful planning, and dedicated support for success.

What we learnt

- Current practice**
- A few other HTA organisations reported currently developing multi-use disease models: CDA (Canada), ICER (USA), ZIN (Netherlands).
- Disease-specific reference case extensions**
- Can be considered an intermediate step to reference models and could promote consistency with fewer barriers.
- Stakeholder Engagement**
- Engaging stakeholders from a variety of backgrounds is critical to ensure accuracy, usability and trust (clinical practice, patient advocacy, commissioning, academia and industry).

Box 1 - Terminology

- NICE reference case**
- NICE’s manuals outline the methods that it considers most appropriate for estimating clinical effectiveness and value for money for the NHS. Health economic modelling teams should follow these methods.
- Disease-specific reference case extensions**
- Extensions to the NICE reference case, which specify the characteristics a health economic model should have for a specific disease or condition
- Signals what NICE considers current best practice in how an economic analysis for a particular disease should be designed.
- Disease-specific reference models**
- Executable economic models, that can evaluate a wide set of interventions within a particular disease or condition.
- They are made available to sponsors submitting technologies for assessment, external assessment groups (EAGs) and teams developing models for clinical guidelines.

Box 2 - NICE’s reference model journey

- Some TA models have been re-used or adapted (e.g. rheumatoid arthritis)
- TAs and guidelines employing pre-existing re-usable models (Diabetes, Duchenne Muscular Dystrophy)
- Guidelines based on whole disease models (atrial fibrillation, prostate cancer)
- Pathway pilots – treatment sequence models (kidney and lung cancer)

Box 3 - Next steps

- Develop reference case extensions starting with
 - Obesity³
 - Metabolic Dysfunction-Associated Steatohepatitis (MASH)
- Develop processes to encourage alignment
 - Scoping
 - Submission/report templates
 - Committee discussions.
- Develop other processes
 - Topic selection
 - Maintenance and updating

Box 4 - Key position statements²

- "1. To enable standardised health economic modelling approaches to inform guidance development, NICE will develop reference case extensions in selected disease areas or conditions.
7. When NICE is aware of a model (or models) that meets one of its reference case extensions, it will notify stakeholders.
8. In some circumstances, NICE will consider building its own reference model.
11. Selection of topics for future disease-specific reference case extensions will focus on NICE's high-priority clinical areas.
12. Prioritisation will also consider which areas can gain significant benefit from a prescribed approach. For example, where:
- ...methodological weaknesses or inconsistencies in modelling approaches in existing models, or
 - modelling methods are not yet well established...,
- ...volume of new products being developed in parallel risks inconsistent approaches to modelling."

References

1. Afzali et al. 2013 Med Decis Making. Apr;33(3):325-32

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3. www.nice.org.uk/guidance/indevelopment/gid-pmg10019

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