Disclosures

Pall Jonsson is employed by the National Institute for Health and Care (NICE). He received a travel grant from the PROTEUS consortium.

NICE

Patient-Reported Outcomes: a Health Technology Assessment Perspective

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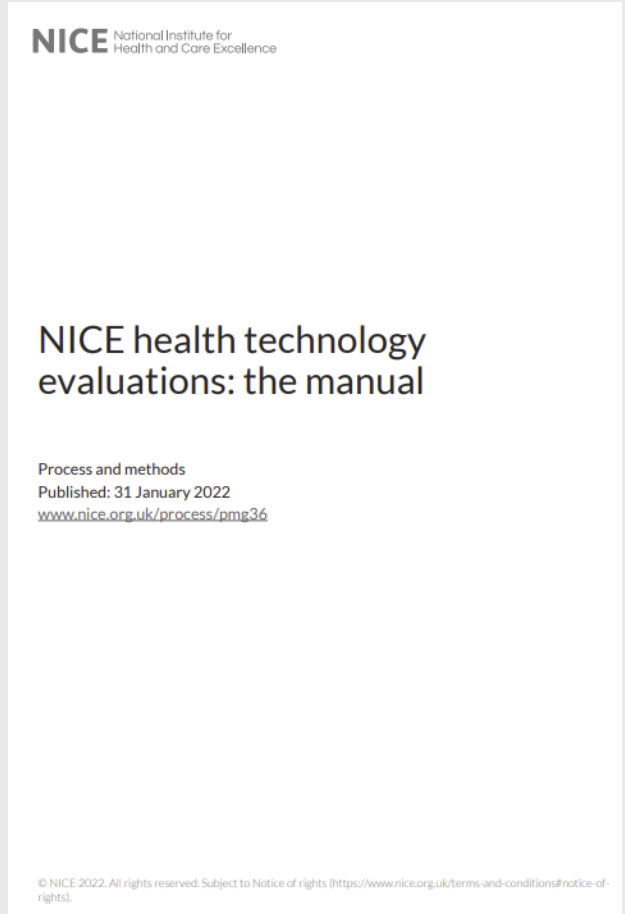
NICE National Institute for Health and Care Excellence

The NICE reference case

NICE has to make decisions across different technologies and disease areas

The reference case specifies the methods considered by NICE in order to maximize health gain from limited resources.

Analyses of clinical and cost effectiveness undertaken to inform the appraisal adopt a consistent approach

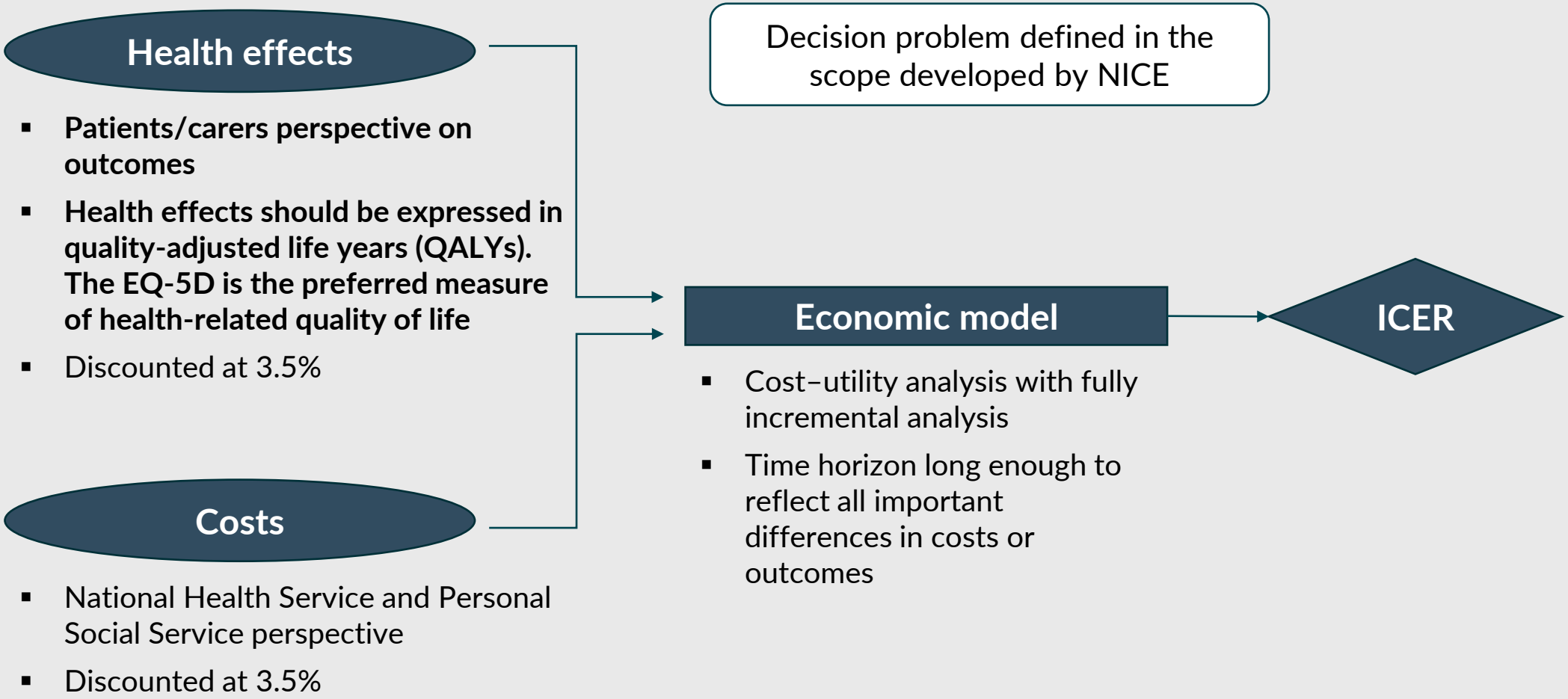

NICE National Institute for Health and Care Excellence

NICE health technology evaluations: the manual

Process and methods
Published: 31 January 2022
www.nice.org.uk/process/pmg36

NICE

Summary of the NICE reference case



NICE

NICE Guide Methods of Technology Appraisal 2022, section 4

There is more to life than EQ-5D


NICE National Institute for Health and Care Excellence

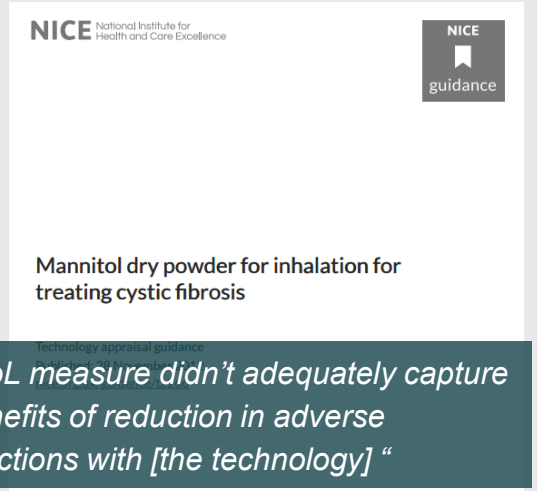
NICE guidance

Adalimumab for treating moderate to severe hidradenitis suppurativa

"improvements in the psychological burden of hidradenitis suppurativa may not be captured in the QALY"

"[the technology] might give enough disease control to allow people to return to work, which has an important positive impact on psychological wellbeing and feelings of self-worth"

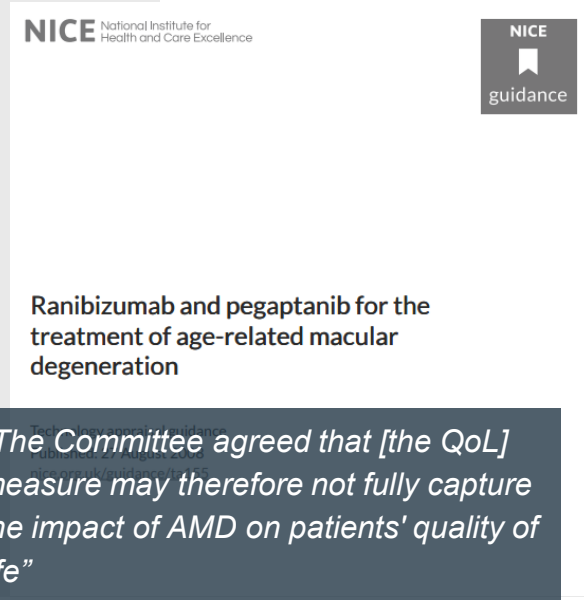
"benefits associated with reducing the wound-care regimen […], such as the time spent on wound care and the effect on work and family life, as well as the cost of dressings, were not captured"


NICE National Institute for Health and Care Excellence

NICE guidance

Mannitol dry powder for inhalation for treating cystic fibrosis

"QoL measure didn't adequately capture benefits of reduction in adverse reactions with [the technology] "


NICE National Institute for Health and Care Excellence

NICE guidance

Ranibizumab and pegaptanib for the treatment of age-related macular degeneration

"The Committee agreed that [the QoL] measure may therefore not fully capture the impact of AMD on patients' quality of life"

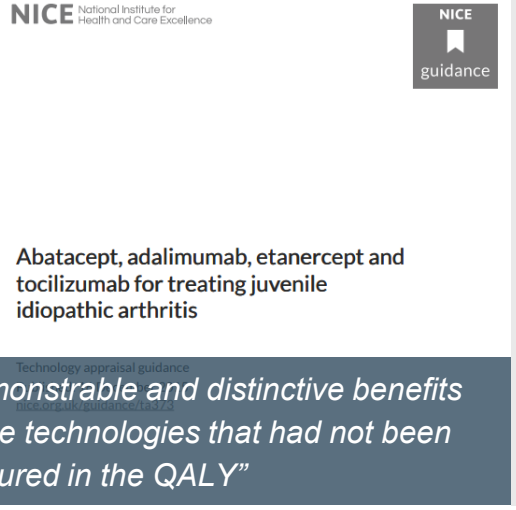

NICE National Institute for Health and Care Excellence

NICE guidance

Mirabegron for treating symptoms of overactive bladder

"The patient experts and clinical specialists also commented that the definition of satisfactory treatment outcomes was broad and varied from person to person, with some people feeling that being completely 'dry' was the only important outcome, whereas others felt that being in control of the symptoms or having fewer micturition episodes was a satisfactory response to treatment."

"EQ-5D would not capture the full utility of treatment"


NICE National Institute for Health and Care Excellence

NICE guidance

Abatacept, adalimumab, etanercept and tocilizumab for treating juvenile idiopathic arthritis

"demonstrable and distinctive benefits of the technologies that had not been captured in the QALY"

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Patient reported outcomes

PROs cover a range of measurement types, including:

- symptom measures (such as pain measured using a Likert scale)
- complex measures (such as activities of daily living or function),
- multidimensional measures (such as health-related quality of life) and
- satisfaction with treatment (patient-reported experience measures, PREMs).

The key component is that the outcome is directly reported by the patient.

Health related quality of life task and finish group report. NICE 2020.
https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/chte-methods-consultation

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NICE's view on PROs

Drugs & MedTech

Digital Health Technologies

1.3.35 In the context of the evaluation, the committee is interested in any limitations in the published research literature identified by patient organisations. In particular, the extent to which patient-reported outcome measures, or other end points reported in clinical studies, capture outcomes that are important to patients. Patients may assess research-based evidence from a different perspective to researchers and clinicians and they may judge the evidence according to different criteria. Also, it is helpful to have the perspective of patients or carers about how relevant the clinical outcomes and the standardised generic instruments for measuring health-related quality of life (as specified in the reference case) are to the disease or condition.

2.2.21 Patient-reported outcome measures can capture important aspects of conditions and interventions such as health-related quality of life, performance status, symptom and symptom burden, and health-related behaviours such as anxiety and depression. They can be either general or disease specific.

NICE health technology evaluations: process and methods manual. Published: January 2022

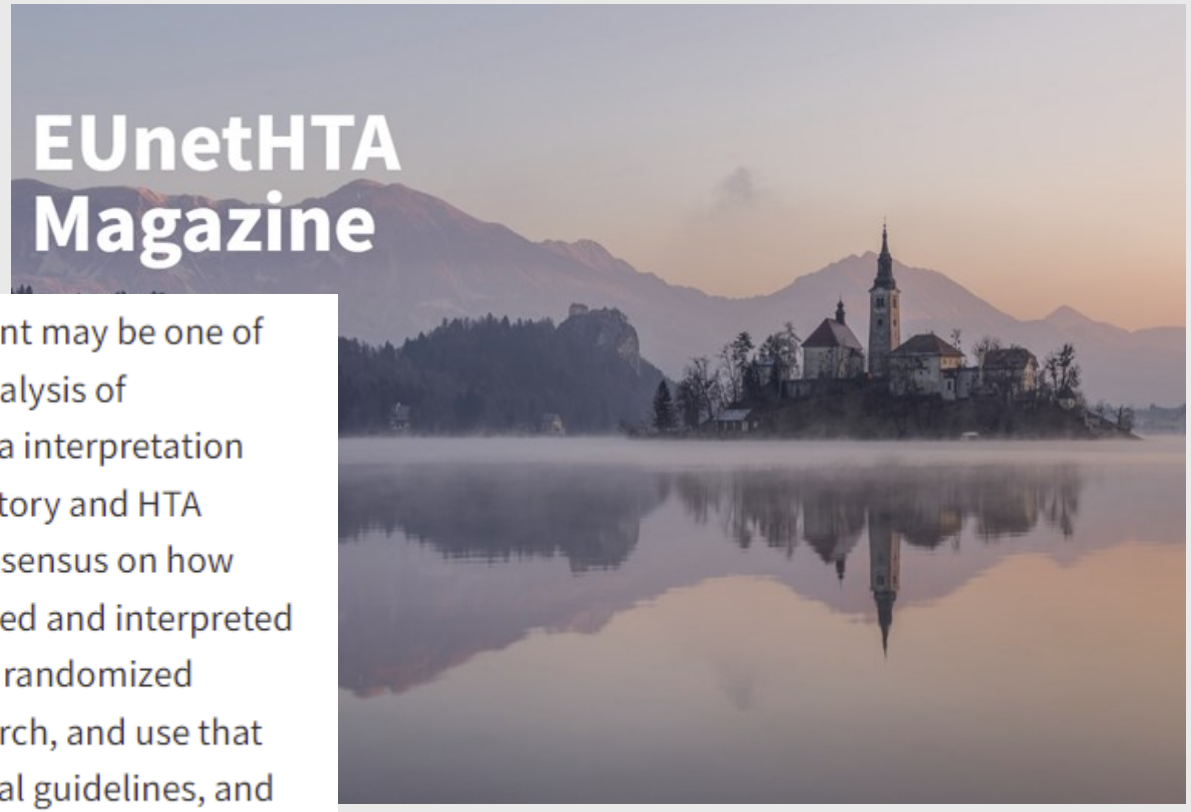
NICE

Table 5 Evidence for effectiveness standards for tier C: intervention DHTs

Evidence category	Minimum evidence standard	Best practice standard
Demonstrating effectiveness for preventative behaviour change or self-manage functions	High-quality observational or quasi-experimental studies demonstrating relevant outcomes. These studies should present comparative data. Comparisons could include: • relevant outcomes in a control group • use of historical controls • routinely collected data.	High-quality intervention study (quasi-experimental or experimental design), which incorporates a comparison group, showing improvements in relevant outcomes, such as: • patient-reported outcomes (preferably using validated tools) including symptom severity or quality of life • other clinical measures of disease severity or disability
Demonstrating effectiveness for treat, active monitoring, calculate or diagnose functions	High-quality intervention study (experimental or quasi-experimental design) showing improvements in relevant outcomes, such as: • diagnostic accuracy • patient-reported outcomes (preferably using validated tools) including symptom severity or quality of life • other clinical measures of disease severity or disability	High-quality randomised controlled study or studies done in a setting relevant to the UK health and social care system, comparing the digital health technology (DHT) with a relevant comparator and demonstrating

Evidence standards framework for digital health technologies. Last updated: April 2021

Beyond NICE: PROs



Lack of standardisation in PRO measurement may be one of the key issues leading to variation in the analysis of results,potentially resulting in different data interpretation and limited impact of these tools on regulatory and HTA decision-making.(3) Indeed, the lack of consensus on how HRQoL and other PRO measures are analysed and interpreted makes it difficult to compare results across randomized controlled trials, synthesize scientific research, and use that evidence to inform product labelling, clinical guidelines, and HTA decisions.

Beate Wieseler, Head of Drug Assessment, IQWiG, Germany
Giovanni Tafuri, Senior Scientific Officer at EUnetHTA, the Netherlands

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Conclusions

NICE, as a HTA body, has a key focus on EQ-5D as a measure of quality of life in health economic assessment

NICE recognises the importance of PROs outside of economic modelling

Most HTA agencies do not yet systematically use PROs to capture the patient's voice

Opportunities for greater use of PROs, especially for digital health technologies

Guidance on quality and methodology is lacking – PROTEUS is an important source of guidance in this space

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