Norwegian Medical Products Agency

The HTA body perspective

11/11/2025; ISPOR Europe; Glasgow

Anja Schiel, PhD, Special Adviser,
Lead Methodologist in Regulatory and Pharmacoeconomic Statistics

- Scientific Advice Working Party member
- Methodology Working Party member
- Former member HTAR-CG JSC and MPG subgroups

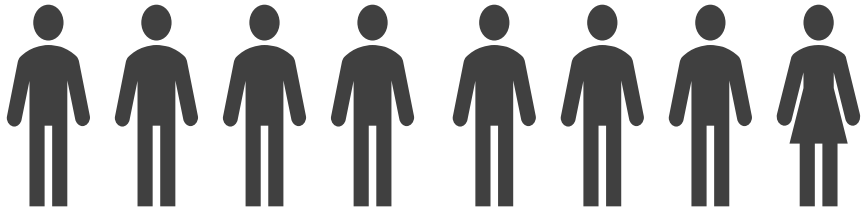
Disclaimer

The views expressed are those of the presenter and should not be understood or quoted as being made on behalf of:

- Norwegian Medical Products Agency (NOMA)
- The European Medicines Agency (EMA) or its scientific committees
- The HTAR Coordination Group (HTAR CG)

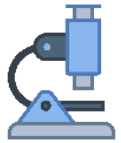
The underlying questions differ

- Clinical trial = Regulator



- Real world = HTA

Efficacy (B/R) = absolute



Effectiveness (C/E) = relative

Does it work in experimental setting

How does it work in medical practice

Population selected

Patients as they come

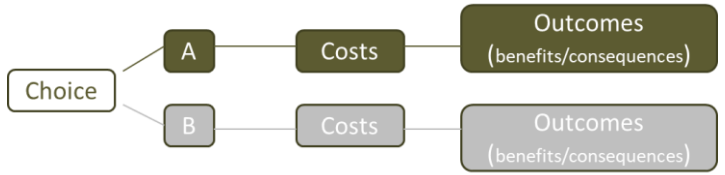
Placebo or a selected comparator

Many alternative treatments

How to get from approval to access

The technology assessment

Clinical evidence with best/high **internal** validity (B/R)



The Health economic decision
To pay or not to pay?
External validity

Generated with ChatGPT/DALL-e

Type	Unit of effect	Strength	Limitations
Cost–benefit analysis (CBA)	All effects measured in €	The net benefit (NB) is easy to interpret. When a new treatments extra benefits are worth more than the extra costs then NB > 0	• It is difficult to measure the value of all health outcomes (positive or negative) in € • Ethical aspects come into the discussion (Prioritisation, discrimination, the Pareto principle)
Cost-utility analysis (CUA)	Two effects (quality and length of life); reflected as quality-adjusted life years (QALY's)	Patient relevant outcomes involving both quality and length of life can be incorporated into the analysis. In theory the QALY measure is 'universal', allowing evaluation of very different decision problems with each other.	• QALY outcomes can be biased by method, indication and population • Society might value a QALY for different patient groups differently (and who should we ask, patients or healthy people form the street?)
Cost-effectiveness (CEA)	Effect measured in 'natural units'	There is one outcome and it is measured in it's 'natural unit'.	• Only one outcome is considered for the effect conclusion (no context)
Cost-minimization (CMA)	No effects measured	Only cost data are needed	• Few treatments have truly identical outcomes • Still some evidence is needed to confirm the assumption of 'equality'

Modified from: Hoch JS, Dewa CS. An Introduction to Economic Evaluation: What's in a Name? *The Canadian Journal of Psychiatry*. 2005;50(3):159-166.

Type	Unit of effect	Strength	Limitations
Cost–benefit analysis (CBA)	All effects measured in €	The net benefit (NB) is easy to interpret. When a new treatments extra benefits are worth more than the extra costs then NB > 0	• It is difficult to measure the value of all health outcomes (positive or negative) in € • Ethical aspects come into the discussion (Prioritisation, discrimination, the Pareto principle)
Cost-utility analysis (CUA)	Two effects (quality and length of life); reflected as quality-adjusted life years (QALY's)	Patient relevant outcomes involving both quality and length of life can be incorporated into the analysis. In theory the QALY measure is 'universal', allowing evaluation of very different decision problems with each other.	• QALY outcomes can be biased by method, indication and population • Society might value a QALY for different patient groups differently (and who should we ask, patients or healthy people form the street?)
Cost-effectiveness (CEA)	Effect measured in 'natural units'	There is one outcome and it is measured in it's 'natural unit'.	• Only one outcome is considered for the effect conclusion (no context)
Cost-minimization (CMA)	No effects measured	Only cost data are needed	• Few treatments have truly identical outcomes • Still some evidence is needed to confirm the assumption of 'equality'

Modified from: Hoch JS, Dewa CS. An Introduction to Economic Evaluation: What's in a Name? *The Canadian Journal of Psychiatry*. 2005;50(3):159-166.

Type	Unit of effect	Strength	Limitations
Cost–benefit analysis (CBA)	All effects measured in €	The net benefit (NB) is easy to interpret. When a new treatments extra benefits are worth more than the extra costs then NB > 0	• It is difficult to measure the value of all health outcomes (positive or negative) in € • Ethical aspects come into the discussion (Prioritisation, discrimination, the Pareto principle)
Cost-utility analysis (CUA)	Two effects (quality and length of life); reflected as quality-adjusted life years (QALY's)	Patient relevant outcomes involving both quality and length of life can be incorporated into the analysis. In theory the QALY measure is 'universal', allowing evaluation of very different decision problems with each other.	• QALY outcomes can be biased by method, indication and population • Society might value a QALY for different patient groups differently (and who should we ask, patients or healthy people form the street?)
Cost-effectiveness (CEA)	Effect measured in 'natural units'	There is one outcome and it is measured in it's 'natural unit'.	• Only one outcome is considered for the effect conclusion (no context)
Cost-minimization (CMA)	No effects measured	Only cost data are needed	• Few treatments have truly identical outcomes • Still some evidence is needed to confirm the assumption of 'equality'

Modified from: Hoch JS, Dewa CS. An Introduction to Economic Evaluation: What's in a Name? *The Canadian Journal of Psychiatry*. 2005;50(3):159-166.

Choose your perspective

Societal perspective

- Medical costs borne by third-party payers and paid out-of-pocket by patients
- Time costs of patients in seeking and receiving care
- Time costs of informal (unpaid) caregivers
- Transportation costs
- Effects on future productivity and consumption
- Other costs and effects outside the health care sector

Health sector perspective

- Include all costs and benefits impacting a system of providers, payers and patients.
- Do not consider impact outside of the health system (e.g. long-term value to patients)
- Based on Direct Medical Costs reimbursed by a third party
- Can include out-of-pocket costs to the patient
- Can include current and future costs as a result of a pathway of care

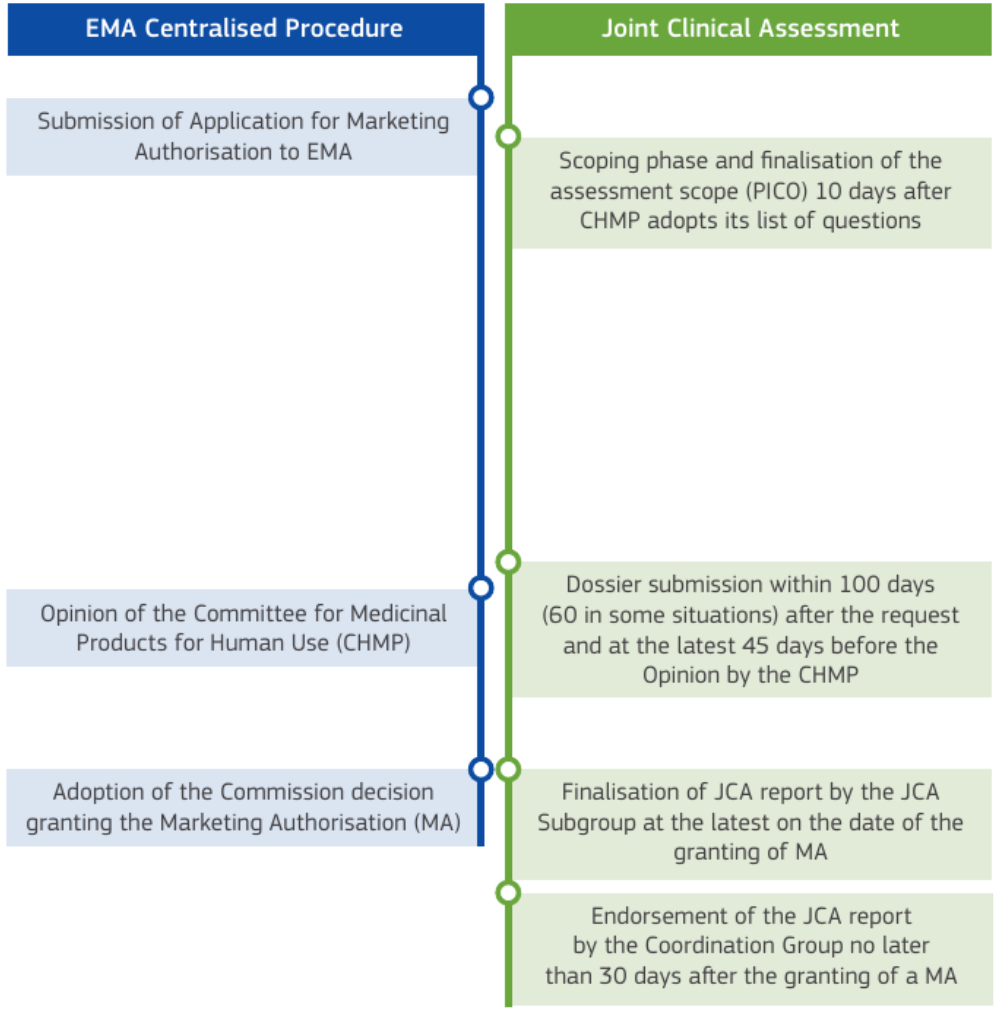
Patient perspective

- Fees for consultation
- Bed day charges at the health facility
- Expenses on medicines, diagnostic tests,
- Travelling expenses to the health facility for the patient and accompanied persons for treatment,
- Amount spent on meal / food taken while waiting for treatment
- Time loss of the patient and the accompanied persons for seeking treatment
- Informal caregiving
- Pain and suffering

Public Payer Perspective

Third-Party Payer Perspective

EMA CENTRALISED PROCEDURE AND JOINT CLINICAL ASSESSEMENT OCCUR IN PARALLELL



Additional timelines of the JCA procedure are detailed in the Procedural guidance for JCA medicinal products
https://health.ec.europa.eu/publications/procedural-guidance-jca-medicinal-products_en

HOW DOES THE PROCESS WORK?

1. START OF JCA PROCESS

1. The **EMA notifies the HTA secretariat** of the receipt of a marketing authorisation application.
2. When submitting a marketing authorisation application to the EMA, the **HTD submits relevant information to the HTA secretariat**.
3. The JCA process formally begins upon the **appointment of assessor and co-assessor** by the JCA subgroup.

2. SCOPING PHASE

4. The assessor and co-assessor draft an **assessment scope proposal** detailing research questions for the JCA. This is known as PICO (Patient or Population Intervention Comparison or Control Outcome).
5. To ensure that the assessment scope reflects the needs of the Member States, the **members of the JCA subgroup are invited to comment** on the suggested scope from their national perspective. In addition, individual experts are invited to provide their input on the assessment scope.
6. The **assessment scope is finalised by the JCA subgroup** and is shared with the HTD in the Commission's initial request for the submission dossier. This is done within **10 days** after the Committee for Medicinal Products for Human Use (CHMP) adopts its list of questions, or at the latest **75 days** after the EMA validation of the marketing authorisation application in accelerated procedures and for variations to the terms of an existing marketing authorisation.
7. The HTD can request an assessment scope explanation meeting with the JCA Subgroup.

3. DOSSIER SUBMISSION

8. The HTD submits a **comprehensive dossier**, including clinical and safety evidence, to the HTA secretariat in digital format within **100 days** of the Commission's initial request (reduced to **60 days** in accelerated procedures and for variations to the terms of an existing marketing authorisation).
9. The assessor and co-assessor can ask the HTD to submit additional information via the HTA secretariat.
10. The HTA secretariat shares all the information received from the HTD with the assessor and co-assessor and the JCA subgroup.

4. DRAFT JCA REPORT

11. The assessor and co-assessor prepare a **draft JCA report and a summary report** with input from the JCA subgroup and the individual experts.
12. The draft JCA and summary reports are **shared with the HTD** to check technical or factual inaccuracies, and to comment on any information the HTD considers to be confidential.
13. The HTD may provide, on their initiative, new relevant information, data, analyses and other evidence to the HTACG via the HTA secretariat.

5. FINALISATION OF JCA REPORT

14. The JCA subgroup **discusses and finalises the draft JCA report** and summary report in a meeting. Individual experts may be invited.
15. The finalised report is submitted to the HTACG for endorsement. The HTACG must endorse the reports no later than **30 days** following the adoption of the Commission Decision granting the marketing authorisation for the medicinal product under assessment.
16. After the Commission's procedural check, the **JCA report and summary report endorsed by the HTACG is published**.

Please note that the Joint Clinical Assessment is to be discontinued, for example, where an application for a marketing authorisation or for a variation to the terms of an existing marketing authorisation is withdrawn, or where the outcome of the centralised procedure is negative.

How to get from approval to access

Scientific Uncertainty

- Development strategy
- Study design elements
  - Sample size (Participants)
  - Follow-up
  - Endpoints
  - Comparator
  - .
  - .
- Lack of contextualisation
- …

Modelling Uncertainty

- Modell Structure
- Assumptions on
  - Population size (Patients)
  - Pre/post treatments
  - True clinical impact
  - Comparator
- Proof of relative effectiveness
- Relevance of relative effectiveness
- …

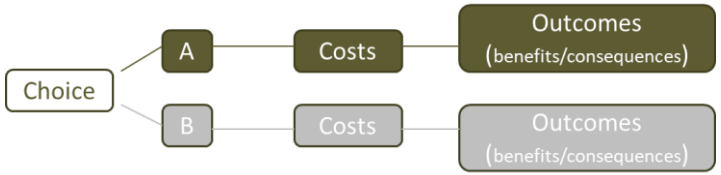
Decision Uncertainty

- Population preferences
- Fairness and Equity
- Affordability / Budget constrains
- System Readiness
- Opportunity costs

- Are we doing no harm?

Where does the JCA come into the picture?

The technology assessment



Clinical evidence with best/high **internal** validity (B/R)

The Health economic decision
To pay or not to pay?
External validity

Generated with ChatGPT/DALL-e

Are we prepared?

- NOMA has a group of dedicated assessors focusing on the different additional activities that came with the HTAR

https://health.ec.europa.eu/health-technology-assessment/implementation-regulation-health-technology-assessment/joint-clinical-assessments_en

Are we prepared? Principle activities, yes

- NOMA has a group of dedicated assessors focusing on the different additional activities that came with the HTAR
  - NOMA has active members in all the HTAR-CG subgroups (JCA, JSC, MPG and Horizon scanning)

Are we prepared? JCA, yes

- NOMA has a group of dedicated assessors focusing on the different additional activities that came with the HTAR
  - NOMA has active members in all the HTAR-CG subgroups (JCA, JSC, MPG and Horizon scanning)
  - NOMA is taking on a first co-assessor role
  - All JCA's require peer review

Name	Indication - Summary	Substance type	Date of EMA validation	Assessor	Co-assessor
Autologous melanoma-derived tumor infiltrating lymphocytes, ex vivo-expanded	Treatment of melanoma	ATMP	3/27/2025	National Authority for Health, France	Agency for Health Technology Assessment and Tariff System, Poland
Tovorafenib	Treatment of paediatric low-grade glioma (LGG)	Chemicals	3/27/2025	National Centre for Pharmacoeconomics, Ireland	Institute for Quality and Efficiency in Health Care, Germany
Sasanlimab	Treatment of bladder cancer	Biologicals	5/22/2025	Dutch National Health Care Institute, The Netherlands	Danish Medicines Council, Denmark
Onasemnogene abeparvovec	Treatment of 5q spinal muscular atrophy (SMA)	ATMP	5/22/2025	National Centre for Pharmacoeconomics, Ireland	National Authority for Health, France
Lurbinectedin	Maintenance treatment of adult patients with extensive-stage small cell lung cancer (ES-SCLC)	Chemicals	6/19/2025	Institute for Quality and Efficiency in Health Care, Germany	National Authority of Medicines and Health Products, Portugal
Camizestrant	Treatment of adults with locally advanced or metastatic breast cancer	Chemicals	6/19/2025	Federation of Social Insurances - Austria	National Institute for Health and Disability (RIZIV-INAMI) - Belgium
Tarlatamab	Treatment of extensive-stage small cell lung cancer	Biologicals	7/17/2025	Institute for Quality and Efficiency in Health Care, Germany	National Centre for Public Health and Pharmacy, Hungary
Catequentinib	Treatment of synovial sarcoma or leiomyosarcoma	Chemicals	7/17/2025	Dental and Pharmaceutical Benefits Agency, Sweden	Norwegian Medical Products Agency, Norway
Senaparib	Maintenance treatment of advanced epithelial high-grade ovarian, fallopian tube or primary peritoneal cancer	Chemicals	8/14/2025	Institute for Quality and Efficiency in Health Care, Germany	Public Agency for Quality in Healthcare, Slovenia

https://health.ec.europa.eu/health-technology-assessment/implementation-regulation-health-technology-assessment/joint-clinical-assessments_en

Are we prepared? JSC, yes

- NOMA has a group of dedicated assessors focusing on the different additional activities that came with the HTAR
  - NOMA has active members in all the HTAR-CG subgroups (JCA, JSC, MPG and Horizon scanning)
  - NOMA is taking on a first co-assessor role
  - All JCA's require peer review

  - NOMA has been involved in all forms of JSC's/parallel advices since 2016
  - Format has changed over time

Are we prepared? In the ‘Nordics’, yes

- NOMA has a group of dedicated assessors focusing on the different additional activities that came with the HTAR
  - NOMA has active members in all the HTAR-CG subgroups (JCA, JSC, MPG and Horizon scanning)
  - NOMA is taking on a first co-assessor role
  - All JCA’s require peer review

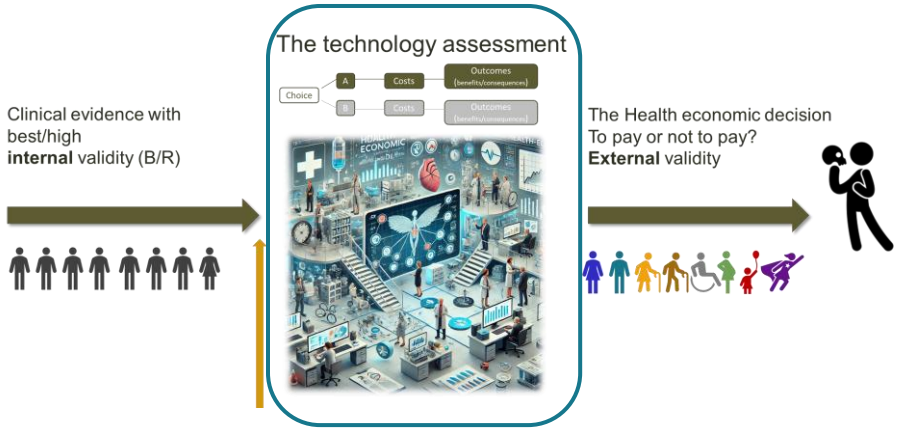
  - NOMA has been involved in all forms of JSC’s/parallel advices since 2016
  - Format has changed over time

- JNHB (formerly FINOSE)
  - All member countries are represented in the HTAR-CG and subgroups (NO,SE, FI, DK and IS)
  - Joined JNHB assessments can include JCAs as submission documentation (same as at national level)

JNHB
Joint Nordic HTA-Bodies

Are we prepared? In the ‘Nordics’, yes

- NOMA has a group of dedicated assessors focusing on the different additional activities that came with the HTAR
  - NOMA has active members in all the HTAR-CG subgroups (JCA, JSC, MPG and Horizon scanning)
  - NOMA is taking on a first co-assessor role
  - All JCA’s require peer review
  - NOMA has been involved in all forms of JSC’s/parallel advices since 2016
  - Format has changed over time



- JNHB (formerly FINOSE)
  - All member countries are represented in the HTAR-CG and subgroups (NO,SE, FI, DK and IS)
  - Joined JNHB assessments can include JCAs as submission documentation (same as at national level)

JNHB
Joint Nordic HTA-Bodies