European Health Technology Assessment: Historical Success of European Joint Assessments

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Poster no: HTA15

INTRODUCTION

- The European Union (EU) has been working towards the streamlining health technology assessments (HTA) and aims to implement a standardized, pan-European framework for all interventions by 2030, starting in 2025 with oncology drugs and advanced therapy medicinal products[1].
- This framework represents the most notable transformation within the European healthcare market in recent times.
- All member states will be required to use this joint clinical assessment (JCA) framework to assess clinical evidence of new interventions. Economic assessment will continue to be conducted at a national level (**Figure 1**) as will recommendations on pricing and reimbursement[2].

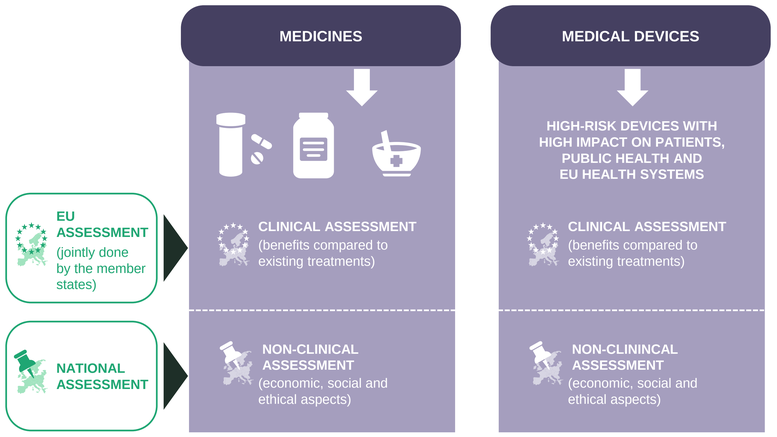
OBJECTIVE

- This aim of this study was to assess the uptake and use of historical voluntary European joint HTA initiatives

METHODS

1. We reviewed European Network for Health Technology Assessment (EUnetHTA) joint assessments of medicines between 2016 and 2021 evaluating involvement and use by European HTA agencies[3].
2. We selected case studies of three medicines with detailed information on use of EUnetHTA assessments, focusing on the UK, Germany, Italy, France and Spain[4].
3. Finally, we assessed the UK's National Institute for Health and Care Excellence (NICE) technology appraisals (www.nice.org.uk) to estimate time between completion of European assessments and NICE published recommendations.

Figure 1. JCA framework – EU compared to national assessment (adapted from European Commission[2])



RESULTS

- There were 20 EUnetHTA assessments (**Table 1**).
- On average, six HTA agencies were involved in the development (3 to 11) and 12 agencies used the assessment reports (3 to 19).
- In the three case studies, where national HTA agencies reported use of EUnetHTA JCAs in national assessments, they were used as background information and cited for reference. There were two cases where the JCA was adapted nationally (**Table 2**).
- NICE carried out a technology appraisal (TA) on half of the medicines, with a mean time from EUnetHTA assessment to NICE assessment of around 9 months (4 to 14) (**Figure 2**). All the medicines were initially recommended by NICE, the majority of which had an accompanying patient access scheme.
- Of those medicines that did not go through a NICE TA, one went through a new health technology evaluation process, four were discontinued due to no information from the company, four were not appraised (of which three were for COVID-19) and one was an appraisal awaiting development.

Table 1. EUnetHTA JCAs 2016 to 2021 for pharmaceutical technologies

No.	Medicine	Indication
PTJA01	Midostaurin	Acute myeloid leukemia
PTJA02	Regorafenib	Hepatocellular carcinoma (previously treated with sorafenib treatment)
PTJA03	Alectinib	ALK+ advanced non-small cell lung cancer (first line)
PTJA04	Sotagliflozin	Type 1 diabetes mellitus (inadequate blood glucose control using insulin or insulin analogues)
PTJA05	Enasidenib	Relapsed or refractory acute myeloid leukemia with an isocitrate dehydrogenase 2 mutation
PTJA06	Polatuzumab vedotin + bendamustine & rituximab	Relapsed/refractory diffuse large B-cell lymphoma (not candidates for hematopoietic stem cell transplant)
PTJA07	Ustekinumab	Moderately to severely active ulcerative colitis
PTJA08	Siponimod	Secondary progressive multiple sclerosis (active disease evidenced by relapses or imaging features of inflammatory activity)
PTJA09	Brolucizumab	Neovascular (wet) age-related macular degeneration
PTJA10	Crizanlizumab	Prevention of vaso-occlusive crises in sickle cell disease patients aged 16 years and over
PTJA11	Cefiderocol	Infections due to aerobic Gram-negative bacteria (limited treatment options)
PTJA12	Glasdegib	Acute myeloid leukaemia (de novo or secondary)
PTJA13	Satralizumab	Neuromyelitis optica spectrum disorders (anti-aquaporin-4 IgG seropositive)
PTJA14	Pretomanid + bedaquiline + linezolid	Pulmonary extensively drug resistant, or treatment-intolerant or nonresponsive multidrug-resistant tuberculosis
PTRCR15	Remdesivir	Covid-19
PTJA16	Venetoclax + hypomethylating agent	Acute myeloid leukaemia (newly diagnosed and ineligible for intensive chemotherapy)
PTJA17	Elivaldogene autotemcel	Cerebral adrenoleukodystrophy
PTRCR18	Dexamethasone	Covid-19
PTRCR19	REGN-Cov2	Covid-19
PTRCR20	Bamlanivimab	Covid-19

Figure 2. Months between EUnetHTA published assessment and NICE TA recommendation

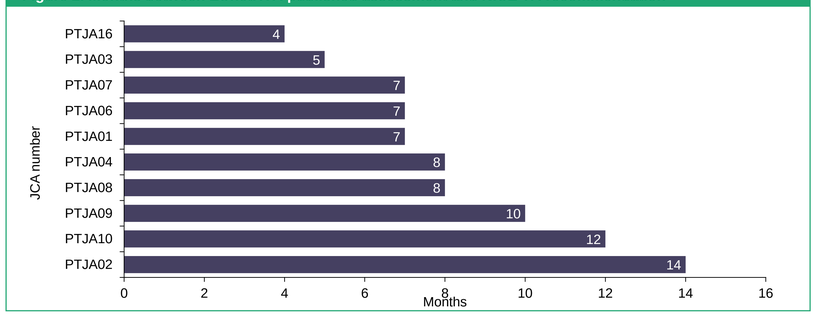


Table 2. Case studies. Use of EUnetHTA JCAs in country specific HTA submissions

Country (HTA)	PTJA01: Midostaurin	PTJA02: Regorafenib	PTJA03: Alectinib
France (HAS)	• JCA cited in the national assessment as background or additional information	• National HTA submission structure partly based on the JCA • JCA cited in the national assessment as background or additional information • HTA committee had a copy of the JCA prior to the national assessment	✗
Italy (AIFA)	• JCA used for background information in national assessment	• Efficacy and safety information in JCA used as supportive information in national report • JCA cited in the national assessment as background or additional information	✗
Italy (UCSC)	✗	✗	• JCA was adapted for national assessment using local information
Spain (AEMPS)	• JCA used for background information in national assessment	• JCA used for background information in national assessment	• JCA used for background information in national assessment
Spain (AETSA)	• English (full report) and Spanish (summary report) versions of the JCA published on AETSA's website for dissemination and distributed to regional decision-makers and hematologists	• English (full report) and Spanish (summary report) versions of the JCA published on AETSA's website for dissemination and distributed to regional decision-makers and oncologists	• JCA adapted for national assessment with no changes to the information
UK (NICE)	• JCA indexed in NHS Evidence	• JCA used as background information to check that similar issues were being identified in national assessment • JCA indexed in NHS Evidence	• JCA used as background information to check that similar issues were being identified in national assessment • JCA indexed in NHS Evidence
Germany (GBA)	✗	✗	✗

Key: ✗, no reported use

CONCLUSION

- Engagement in and use of a previous joint HTA initiative was varied among European HTA agencies, reflecting differences in national HTA requirements, which may be due to different national scope requirements for population, intervention, comparator, and outcome (PICO) and the need for an economic assessment for some national HTA agencies.
- The upcoming standardized JCA framework aims to facilitate timely patient access by reducing duplication of assessment efforts.
- To achieve this, transparent and detailed process and method guidelines are required from the forthcoming implementing acts, particularly in relation to scoping and dossier requirements.

Abbreviations

AEMPS, Agencia Española de Medicamentos y Productos Sanitarios [Spanish Agency of Medicines and Medical Devices]; AETSA, Agencia de Evaluación de Tecnologías Sanitarias de Andalucía [Andalusian Agency for Health Technology Assessment]; AIFA, Agenzia Italiana del Farmaco [Italian Medicines Agency]; EU, European Union; EUnetHTA, European Network for Health Technology Assessment; GBA, Gemeinsame Bundesausschuss [German Federal Joint Committee]; HAS, Haute Autorité de Santé [French National Authority for Health]; HTA, health technology assessment; JCA, joint clinical assessment; NICE, National Institute for Health and Care Excellence; PICO, population, intervention, comparator, and outcome; TA, technology appraisal; UCSC, Università Cattolica del Sacro Cuore

References

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ISPOR, Atlanta, USA; 5 – 8 May 2024

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