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HTA in paediatric research

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HTA Regulation

‘health technology assessment’ or ‘**HTA**’ means a multidisciplinary process that summarises information about the medical, patient and social aspects and the economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased and robust manner.

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REGULATION (EU) 2021/2282 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL of 15 December 2021 on health technology assessment and amending Directive 2011/24/EU

HTA Regulation

‘**health technology**’ means a medicinal product, a medical device or medical and surgical procedures as well as measures for disease prevention, diagnosis or treatment used in healthcare.

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HTA-R and DIRECTIVE 2011/24/EU OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL of 9 March 2011 on the application of patients’ rights in cross-border healthcare

HTA Regulation

- The development of health technologies is a **key driver** of economic growth and innovation in the EU
- **Health technologies** constitute an innovative sector of the economy and form part of an overall market for healthcare expenditure that **accounts for 10 % of Union gross domestic product**
- HTA is a **scientific evidence-based process** that allows competent authorities to determine the **relative effectiveness** of new or existing health technologies. HTA focuses specifically on the **added value** of a health technology in comparison with other new or existing health technologies
- HTA is able to contribute to the **promotion of innovation**

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HTA Regulation

The Regulation establishes:

a) a **support framework and procedures for cooperation** of Member States on health technologies at Union level;

b) a mechanism which lays down that **any information, data, analyses and other evidence** required for the joint clinical assessment of health technologies **is to be submitted by the health technology developer only once** at Union level;

c) common rules and methodologies for the **joint clinical assessment** of health technologies

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- HTA can cover both clinical and non-clinical aspects of a health technology (**9 domains of assessment**):
  - **clinical** (4): Health problems and currently used health technologies (e.g. medicines, medical devices, surgical procedures), description of the health technology under assessment, relative clinical effectiveness, relative safety
  - **non-clinical** (5): cost and economic evaluation of a health technology, and its ethical, organisational, social and legal aspects

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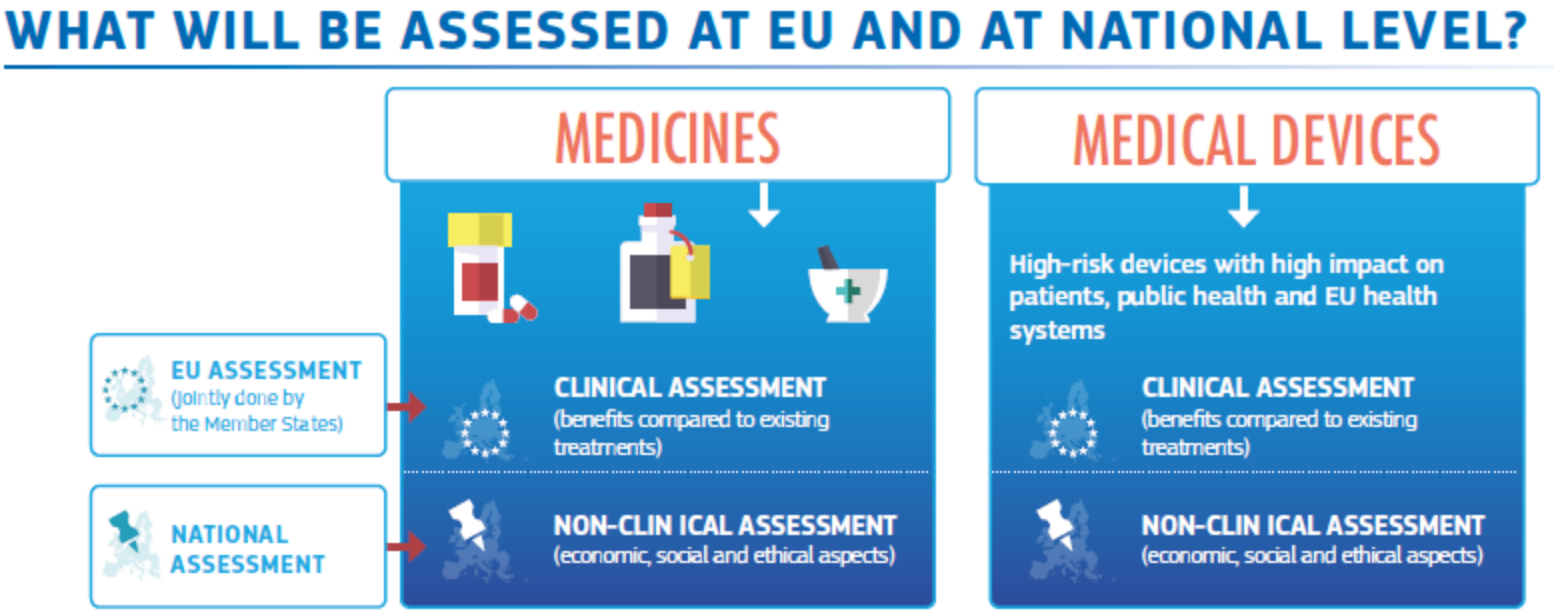
HTA Regulation



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HTA Regulation

TIMELINE FOR MEDICINES

- 12 January 2025: New oncology medicines and advanced therapy medicinal products will be assessed at EU level.
- 13 January 2028: Orphan medicinal products to be added to the joint work.
- 13 January 2030: All new medicines will come under the scope of the regulation.

TIMELINE FOR MEDICAL DEVICES

After 12 January 2025 and at least every two years, medical devices and in vitro diagnostic medical devices selected for joint clinical assessment based on:

a) unmet medical needs;
b) first in class;
c) potential impact on patients, public health or healthcare systems;
d) incorporation of software using artificial intelligence, machine learning technologies or algorithms;
e) significant cross-border dimension;
f) major Union-wide added value

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Joint clinical assessment reports and the dossier of the health technology developer

JCA reports shall be based on a dossier that contains complete and up-to-date information, data, analyses and other evidence submitted by the health technology developer to assess the parameters included in the assessment scope.

The dossier shall meet the following **requirements**:

- the submitted evidence is complete with regard to the available studies and data that could inform the assessment;
- the data has been analysed using appropriate methods to answer all research questions of the assessment;
- the presentation of the data is well structured and transparent, thereby allowing for an appropriate assessment within the limited timeframes available;
- it includes the underlying documentation in respect of the submitted information, thereby allowing the assessor and co-assessor to verify the accuracy of that information.

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HTA and Paediatrics

Received: 25 May 2021 | Revised: 29 November 2021 | Accepted: 3 December 2021
DOI: 10.1111/bcp.15190

THEMED ISSUE REVIEW

Health technology assessment of paediatric medicines: European landscape, challenges and opportunities inside the conect4children project

Francesco Moretti[1] | Fabrice Ruiz[2] | Fedele Bonifazi[3] | Elena Pizzo[4] | Jenny M. Kindblom[5] | the c4c HTA expert group

… access to therapies is heavily influenced by national HTA recommendations, which often form the basis for pricing and reimbursement decisions …

In 2007-2020:

- **325 paediatric medicines** approved by the EMA
- **176** authorised according to the provisions of the **Paediatric Regulation**
- 149 are paediatric generics, biosimilars or hybrid products
- **< 40%** of 176 with a positive match in the international HTA database by INAHTA (International Network of Agencies for HTA) hosting 17 656 records of bibliographic information about ongoing and published HTAs, and **full HTAs are only a minority**

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DOI: 10.1111/bcp.15190

HTA and Paediatrics

Poster #HTA241

Health Equity and the Health Technology Assessment Process: Are Children and Young People Being Overlooked? A Review of Pediatric National Institute of Health and Care Excellence (NICE) Technology Appraisals

Donata Freigofaite[1], Angeliki Kaproulia[1], Andre Verhoek[1], Grammati Sarri[2]
[1]Cytel, Inc., Rotterdam, Netherlands; [2]Cytel, Inc., London, UK

Technologies targeting children and young people constitute a small minority (**<5%**) of total technologies assessed every year by the National Institute for Health and Care Excellence (**NICE**). Current HTA methods and approaches do not largely account for the unique realities of evidence generation and synthesis in child health and disease experience.

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DOI: https://doi.org/10.1016/j.jval.2023.09.1924

HTA and Paediatrics

Mascarenhas *et al. Cost Effectiveness and Resource Allocation* (2024) 22:33
https://doi.org/10.1186/s12962-024-00537-0

Cost Effectiveness and Resource Allocation

REVIEW Open Access

Economic evaluations of medical devices in paediatrics: a systematic review and a quality appraisal of the literature

Edgar Mascarenhas[1*], Luís Silva Miguel[2], Mónica D Oliveira[1,3] and Ricardo M Fernandes[4,5]

… economic evaluations (EEs) have been increasingly applied to medical devices, … **most EEs on devices are conducted in adults**, with specific aspects related to the uniqueness of child health often being overlooked.

… results highlight the **need to improve the quality of reporting** and advance methods that can explicitly incorporate the multiple impacts related to the use of devices with distinct characteristics, as well as **consider specific child health realities**.

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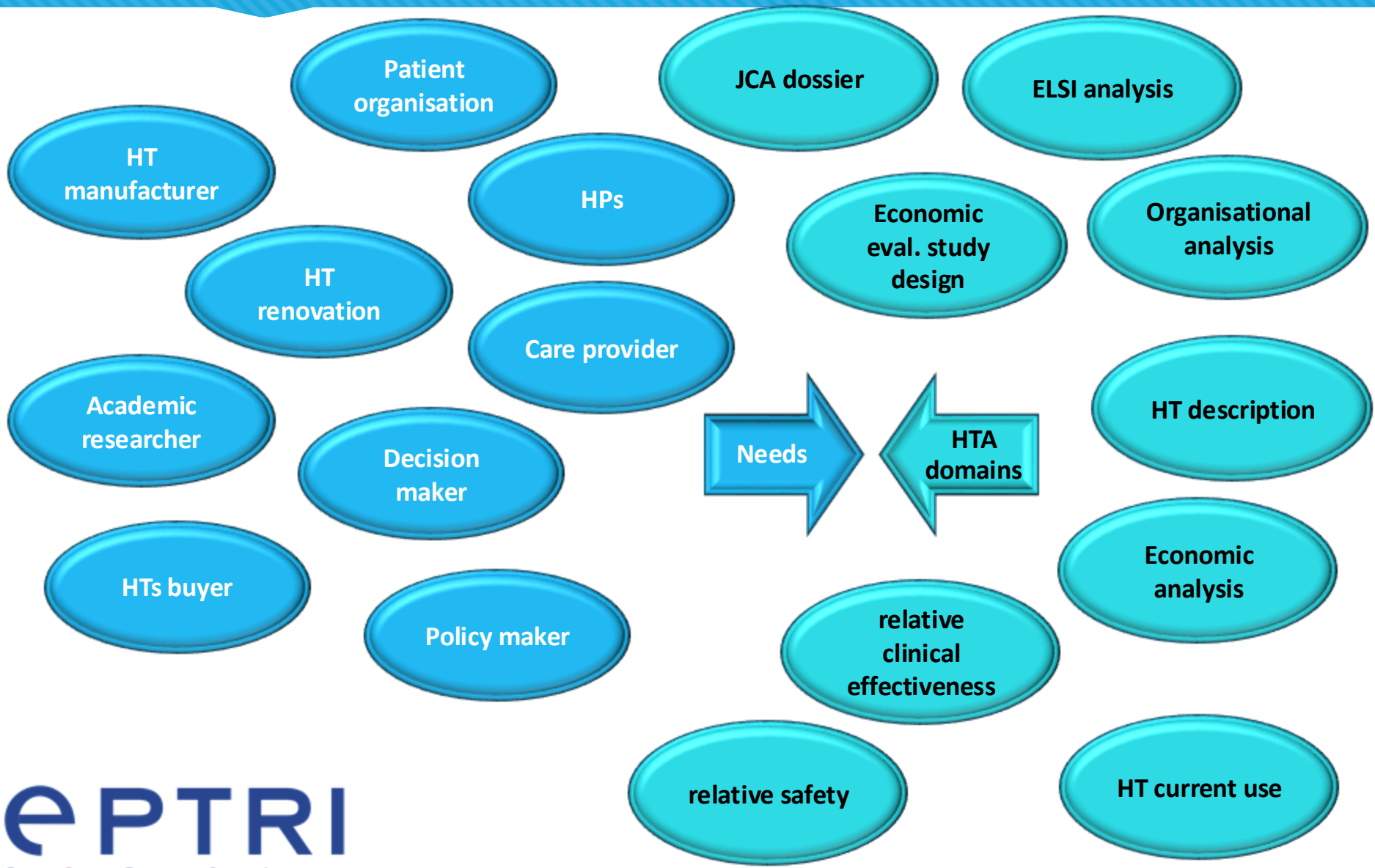
https://doi.org/10.1186/s12962-024-00537-0

Paediatric HTA as a service



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