



Bundesministerium  
für Gesundheit

# EU HTA Regulation

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Dr. Lars-Christoph Nickel, BMG

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# I. Legislative Process

# I. Legislative Process (1)

- On January 31st, 2018 the European Commission (EC) made a proposal for a EU HTA regulation based on Article 114 TFEU:
  - Harmonisation of Health Technology Assessment (HTA) for all newly approved medicinal products (pharmaceuticals) and certain medical devices
  - Mandatory use of „Joint Clinical Assessment“ (JCA) by Member States (MS)
  - Prohibition to conduct national HTA procedures
- Due to the obligatory design for the MS, the German Bundestag (BT) filed a subsidiarity complaint, the German Bundesrat (BR) has raised similar concerns
- Also, France and the Czech Republic filed a a subsidiarity complaint

# I. Legislative Process (2)

- The European Parliament (EP) has decided on a position on the proposal on February 14th, 2019
- The negotiations in the European Council (Council) were stuck due to complex competence issues
- After more than two years of difficult negotiations, the German Council presidency presented a compromise integral text in late 2020
- Due to the challenges of the COVID-19-pandemic, German Council presidency was not able to finalize the negotiations
- Based on the German proposal, Portuguese Council presidency received the mandate to open negotiations with the EP on March 24th, 2021

# I. Legislative Process (3)

- On June 21st, 2021 a provisional agreement was reached between EP and Council
- Provisional agreement was confirmed by MS at COREPER on June 30th, 2021; ENVI committee at EP has agreed on July 13th, 2021
- Council expected to vote on finalised Text in November 2021 and EP in Dezember 2021
- Subsequent publication in the EU official journal and entry into force of EU HTA regulation in winter 2021/2022

## II. Cornerstones of the EU HTA Regulation

## II. Cornerstones of the EU HTA Regulation (1)

- Based on Art. 114 (Common Market) and Art. 168 (Health) TFEU the Regulation establishes:
  - a support framework and procedures for cooperation of MS on health technologies at EU level
  - a mechanism stipulating that any information, data, analyses and other evidence required for the JCA is submitted by the health technology developer (developer) **only once** at EU level (Article 114 TFEU);
  - common rules and methodologies for the JCA of health technologies
- The Regulation shall not affect MS' competence to draw conclusions on the relative effectiveness of health technologies and to take decisions on the use of a health technology in their specific national health context



## II. Cornerstones of the EU HTA Regulation (2) Cooperation of MS on health technologies

Focus of cooperation between the MS on health technologies at EU-level:

1. Joint Clinical Assessments for medicinal products and certain medical devices
2. Joint scientific consultations for manufacturers (e.g. on the design of clinical trials, parallel consultations HTA/EMA)
3. Horizon scanning (early identification of emerging health technologies that are likely to have a major impact on patients, public health and healthcare systems)
4. Voluntary cooperation in other areas (e.g. non-clinical aspects of HTA or other health technologies)

## II. Cornerstones of the EU HTA Regulation (3)

### Cooperation of MS on Joint Clinical Assessments (JCA):

- MS agree to cooperate and develop JCAs
- JCAs create a sound scientific foundation for pricing and reimbursement decisions for all MS
- MS remain responsible for drawing conclusions at national level on the clinical added value of a health technology, but also shall “give due consideration” to the JCAs
- Assurance of a high quality and a fixed time frame for JCAs
- Ensurance of the highest possible level of transparency regarding the submitted data
- **Exclusive competence of MS for pricing and reimbursement remains unaffected** (Art. 168 (7) TFEU)

# III. Governance Structure

# III. Governance Structure (1)

Coordination Group of MS

Subgroups of Coordination Group

Joint Assessments

Joint scientific consultations

Horizon Scanning

Methodological and procedural guidance

Stakeholder network

EC as secretariat



# III. Governance Structure (2): Coordination Group of Member States

Central entity is the Coordination Group (CG) of MS:

- MS shall designate their members of the CG, Meetings of the CG shall be chaired and co-chaired by two elected members for a limited set term
- The CG shall carry out its activities in an independent, impartial, in a transparent manner and of the highest quality, follows international standards of evidence-based medicine
- Voting mechanism:  
**Simple majority** vote is generally applied, **but** for decisions on annual work programme, annual report and strategic direction for subgroups a **qualified majority** vote is applied

# III. Governance Structure (3): Subgroups of Coordination Group

The Subgroups prepare the decision of the CG:

- Members of CG assign national authorities or bodies responsible for HTA as members of subgroups
- Regulation envisages the following subgroups:
  - JCAAs
  - Joint Scientific Consultations
  - Identification of emerging health technologies
  - Development of methodological and procedural guidance
- CG can implement additional subgroups

# III. Governance Structure (4): Stakeholder Network

EC establishes a Stakeholder Network that should provide input on JCAs and Joint Scientific Consultations:

- Network should include patient organisations, healthcare professional organisations, developer associations, consumer organisations and other relevant NGO's in the field of health
- Eligibility criteria: demonstrated current or planned engagement in HTA development; professional expertise relevant to the Network; geographical coverage of several MS; communication dissemination capabilities, declaration of conflicts of interests
- CG meets Stakeholder Network at least annually
  - Report of CG on joint work on EU HTA and exchange of information
  - CG may invite members of Stakeholder Network to their meetings as observers

# III. Governance Structure (5): Role of the European Commission

- EC shall act as the Secretariat of the CG and support its work :
  - host on its premises the meetings of the CG and of its subgroups
  - decide on conflict of interest; requests the submission of the dossier and confirms the timely and complete submission of the dossier
  - supervise the procedures for JCAs and inform the CG about possible breaches thereof
  - provide administrative, technical and IT support; set up and maintain the IT platform
  - publish the information and documents
  - facilitate the cooperation, notably through the exchange of information with the European Medicines Agency and expert panels
  - adoption of implementing and delegating acts
- No later than three years after the date of application, EC shall present a report to the EP and the Council on the application of this Regulation (winter 2027/2028)



# III. Governance Structure (6): Funding of EU HTA Cooperation

- The financing of the cooperation will be ensured by the EU and will cover, inter alia:
  - The work of the CG and its subgroups including the participation of the CG members at the CG's meetings to support the work on the JCAs
  - Cooperation with EC, EMA and Stakeholders
  - Development of methodological guidelines
  - Assessor and Co-Assessor are entitled to a special allowance for their work on JCAs and Joint Scientific Consultations in accordance with internal EC rules

## IV. Scope of Application

# IV. Scope of Application (1): Medicinal Products

The regulation will apply three years after entry into force. For medicinal products it stipulates a stepwise approach :

- Step 1: with the date of application of the Regulation, for medicinal products with new active substances for which the therapeutic indication is the treatment of cancer and for ATMPs (winter 2024/2025)
  - Step 2: Three years after the date of application of the Regulation for medicinal products which are designated as orphan medicinal products (winter 2027/2028);
  - Step 3: Five years after the date of application of the Regulation, for all medicinal Products (winter 2029/2030)
- Upon recommendation of the CG, EC may select further medical devices and in-vitro diagnostic medical devices for JCA (e.g. for „Unmet medical need“, „public health emergency“, „significant impact on systems“)

## IV. Scope of Application (2): Medical Devices

After the date of application of this Regulation (winter 2024/2025), EC shall select the medical devices and in-vitro diagnostic medical devices for JCA based on one or more of the following criteria:

- „unmet medical needs“
  - „first in class“
  - „potential impact on patients, public health or health systems“
  - „incorporating software using artificial intelligence, machine learning technologies or algorithms“
  - „significant cross-border dimension“
  - „major EU-wide added value“
- After seeking recommendation of the CG and by way of implementing act

# V. Process of the EU HTA Assessments

# V. Process of the EU HTA Assessments (1): Initiation of Assessment

The CG shall initiate JCAs of health technologies by designating the sub-group on JCAs

- sub-group appoints an assessor and a co-assessor
- sub-group initiates a scoping process, that reflects all MS' needs in terms of parameters and information („Patient Population“, „Interventions“, „Comparators“, „health outcome“ etc.)
- CG shall inform the EC of the assessment scope of the JCA
- EC informs the developer of the assessment scope and requests the submission of the dossier:
  - Defined procedural steps and timing will be set in rules of procedure of CG
  - For medicinal products, the deadline for submission shall be at the latest 45 days prior to the envisaged date of the opinion of the Committee for Medicinal Products for Human Use (CHMP)

# V. Process of the EU HTA Assessments (2): Requirements on Dossier

- minimum requirements for dossier are listed in regulation:
  - the submitted evidence shall be complete with regard to the available studies and data that could inform the assessment
  - the data shall be analysed using appropriate methods to answer all research questions of the assessment
  - the data presentation shall be well-structured and transparent to allow for an appropriate assessment within the limited timeframes available and to support the understanding of the submission and the assessment by third parties
  - it shall include underlying documentation of the information presented to allow the assessors to verify the accuracy of the submitted information



Details in Annex I and II of EU HTA Regulation

# V. Process of the EU HTA Assessments (3): Obligations of Health Technology Developers

- The developer shall submit the dossier in the set timeframe. EC confirms the timely submission of the dossier and that it meets the formal requirements. EC makes the dossier available to the members of the CG.
- Where EC finds that the dossier fails to meet the formal requirements, it shall request the missing information, data, analyses and other evidence from the developer (second request)
- If dossier still fails to meet the formal requirement after the second request, the CG discontinues the JCA; re-initiation is possible only after at least 6 months
- The developer shall not submit any information, data, analyses or other evidence at the national level that has been already submitted at EU level; Exception: early access programmes at Member State level



# V. Process of the EU HTA Assessments (4): JCA Report

- As the result of the JCA a report (including a summary report) will be published
- JCA Report will not draw conclusions on the overall clinical added value of a health technology and is limited to a descriptive scientific analysis of:
  - The relative effects of health technology on relevant clinical outcomes of health technology as assessed on the health outcomes against the chosen parameters based on the assessment scope
  - the degree of certainty on the relative outcomes, based on the strengths and limitations of the available evidence
- Adoption of JCA report for medicinal product at the latest one month after EU approval; no rule for medical devices implemented in regulation

# V. Process of the EU HTA Assessments (5): Preparation of draft JCA Report

On the basis of the dossier submitted by the developer and the assessment scope the assessor, with the assistance of the co-assessor, shall prepare the draft JCA report:

- members of the subgroup and stakeholders are being given an the opportunity to provide input on the draft report
- the developer is given the opportunity to comment on technical or factual inaccuracies
- Following receipt and consideration of comments the assessor, with the assistance of the co-assessor, shall prepare revised draft report, and submit those to the CG

# V. Process of the EU HTA Assessments (6): Finalisation of JCA

- Due to scientific character of the report, CG shall endeavour to endorse the report by consensus. Where consensus cannot be reached, all divergent scientific opinions, shall be incorporated in the report
- The CG submits the report to EC for procedural review:
  - EC publishes the procedurally compliant report and informs the developer of the publication
  - If EC concludes that report does not comply with the procedural rules, it informs the CG and requests a review of the report
  - If EC concludes that the reviewed report still does not comply with the procedural rules it makes the report and its procedural review available solely for the consideration of MS and informs the developer

## VI. Member States' Rights and Obligations

# VI. Member States' Rights and Obligations (1)

- MS shall „give due consideration” to the published JCA report concerning that JCA in their health technology assessments at Member State level
- This shall not affect MS' competence to draw their conclusions on the overall clinical added value of a health technology in the context of their specific healthcare system and to consider the parts of the report relevant in this context
- MS shall annex the published JCA report to the health technology assessment report at Member State level
- MS not request at the national level information or data that has been submitted by the developer at EU level
- MS shall immediately share any information or data with the CG that they receive from the developer at Member State level

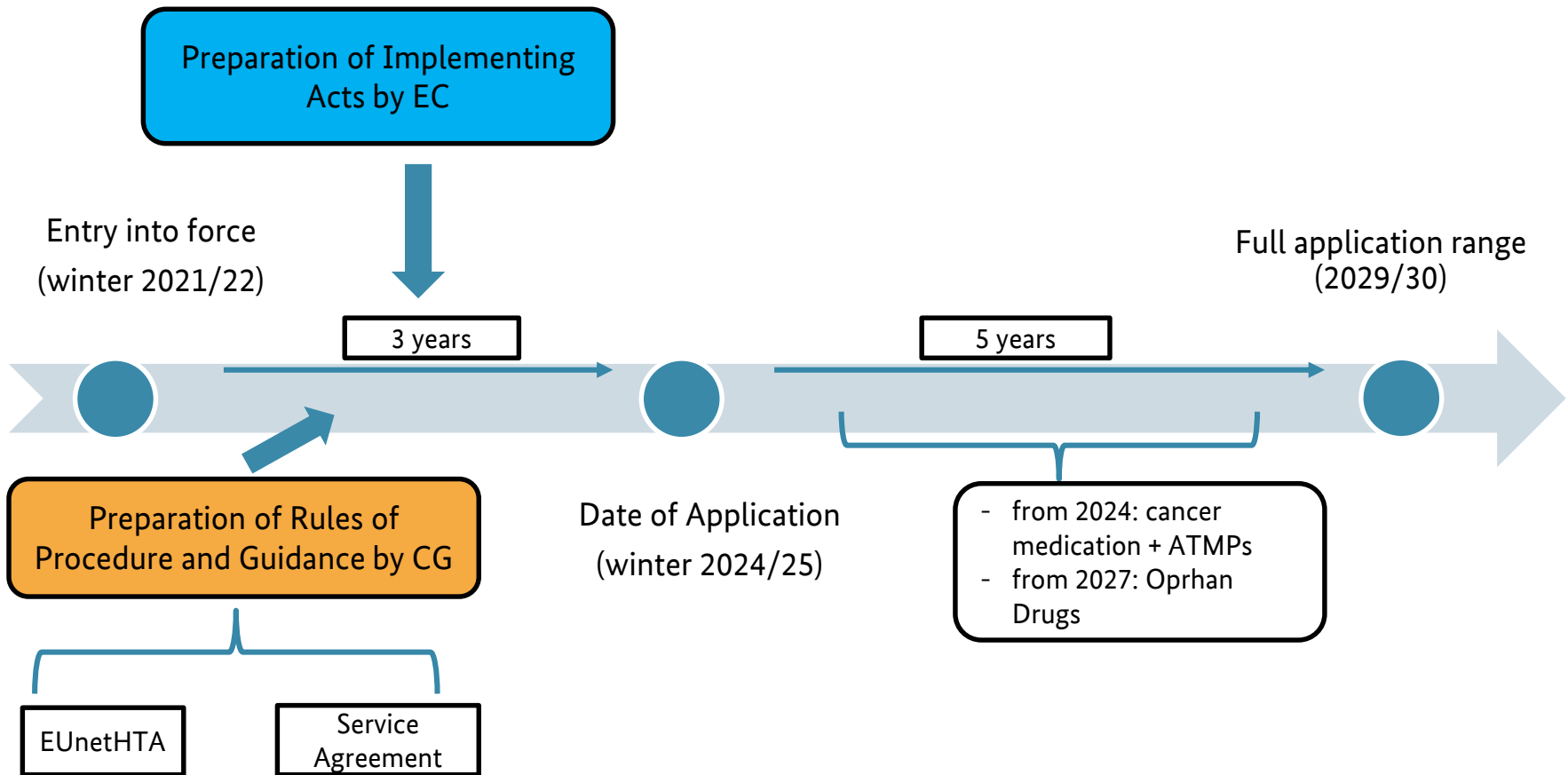
# VI. Member States' Rights and Obligations (2)

- MS shall provide CG with information on national HTA which has been subject to a JCA within 30 days:
  - In particular, MS should provide information on how JCA report have been considered when carrying out national HTA
  - EC will publish annual report of uptake of report in MS, based on this information
- No later than two years after the date of application (winter 2026/2027), MS shall report to EC on the implementation of the Regulation, including
  - the way JCAs have been considered when carrying out national HTA
  - whether they have considered methodological guidance

This information will be the basis for the report of EC to EP and Council in winter 2027/2028

## VII. Implementation of EU HTA Regulation

# VII. Implementation of EU HTA Regulation: Timeline





# VII. Implementation of EU HTA Regulation (2): Legal Acts of EC

EC prepares until Date of Application the following Implementing Acts:

- Rules of Procedure for cooperation with EMA/notified bodies/ expert panels and Stakeholders for JCA (Comitology procedure)
- Rules of Procedure for cooperation with EMA/notified bodies/ expert panels and Stakeholders for Joint Scientific Advice (Comitology procedure)
- General Rules of Procedure for:
  - Independence, transparency, and avoidance of conflicts of interest of all involved parties
  - the selection and consultation of stakeholder organisations and patient organisations, clinical and other relevant experts in JCAs (Comitology procedure)
- Implementing Acts establishing the format and templates for dossiers and JCA report (Comitology procedure)

# VII. Implementation of EU HTA Regulation (3): Preparational Work Coordination Group

CG prepares until Date of Application the following:

- General Rules of Procedure
- Methodological Guidance on joint work following international standards of evidence-based medicine
- Rules of Procedure for detailed procedural steps and their timing for JCAs and for updates of JCAs
- Rules of Procedure for detailed procedural steps and their timing for Joint Scientific Consultations, including submissions of request from developers
- Procedures that ensure the highest quality, follows international standards of evidence-based medicine of JCA report
- Format and templates of requests for joint scientific consultations, dossiers for joint scientific consultations and joint scientific consultation outcome documents

## VII. Excursion- Revision of the EU- Legal Framework for Medicinal Products and Medical Devices

# VII. Excursion- Revision of the EU- Legal Framework for Medicinal Products and Medical Devices (1) - Pharmaceutical Strategy for Europe

- EC published the „Pharmaceutical Strategy for Europe“ November 25, 2020
- Objectives:
  - Improving access to innovative and affordable medicines
  - Supporting the competitiveness and innovation of the EU pharmaceutical industry
  - Development of an open strategic EU autonomy and ensuring a reliable EU pharmaceutical supply
  - Ensuring a strong EU voice in the international environment

# VII. Excursion- Excursion- Revision of the EU- Legal Framework for Medicinal Products and Medical Devices (2) -Pharmaceutical Strategy for Europe

- Various needs for action and proposed solutions for the years 2020 to 2024
- 55 legislative and non-legislative flagship initiatives and other measures
- Most important measure: Revision of Directive 2001/83/EC (Community code relating to medicinal products for human use) and Regulation (EC) Nr. 726/2004 (Union procedures for the authorisation and supervision of medicinal products for human use and establishing an European Medicines Agency)
  - First roadmap / first impact assessment of the EC on March 30, 2021
  - Legislative proposal of the EC planned for the 4th quarter of 2022

# VII. Excursion- Revision of the EU- Legal Framework for Medicinal Products and Medical Devices (3) - Medicines for Children and Rare Diseases

## **Regulation (EC) No 141/2000 on orphan medicinal products**

- Goal: creation of incentives for research, development and market authorization of medicines for rare diseases (orphan drugs)

## **Regulation (EC) No 1901/2006 on medicinal products for paediatric use**

Evaluation of the European Legislation on medicines for children and rare diseases completed in August 2020

Inception impact assessment of a revision of the legislation by the EC published in November 2020

# VII. Excursion- Revision of the EU- Legal Framework for Medicinal Products and Medical Devices (4) - Medicines for Children and Rare Diseases

## **Main weakness:**

- Insufficient development of medicines in areas with
  1. unmet medical needs
  2. few chances of profitability for a pharmaceutical company
- mainly in specific areas, like rare diseases in children

## Revision of the Regulations

- investigation of possibilities for more specific incentives in the future
- focus on the definition of and criteria for unmet medical needs concerning medicines for children and for rare diseases

Legislative proposal by the EC announced for first quarter of 2022

# VII. Excursion- Revision of the EU- Legal Framework for Medicinal Products and Medical Devices (5) - Blood and Tissue Directives

Setting of minimum quality and safety standards for blood and blood products and for the distribution of tissues and cells with national provisions in the Transfusion and Transplantation act.

## **Objectives of the revision of the BTC legislation (BTC: Blood, Tissues, Cells)**

- Enabling the best possible protection for patients against avoidable health risks during treatment with blood, blood products, cells and tissues.
- To improve the protection of BTC donors and children born with the help of medically assisted reproduction.
- Improving the trafficability of blood, blood products, cells and tissues in the EU by harmonizing oversight systems for inspections, authorization and vigilance.
- Improving the framework conditions for innovative processing, authorization procedures and new therapy methods.
- Creating legal certainty regarding the scope of application of BTC legislation.
- Reduction of supply dependency on third countries and of resulting supply shortages and ensuring the supply of the population in Europe.



# VII. Excursion- Revision of the EU- Legal Framework for Medicinal Products and Medical Devices (6) - Blood and Tissue Directives

Status of the process:

- Evaluation by EC in 2019
  - as a result after 16 years no longer specific enough
  - does not reflect all new scientific and technical developments
- Inception Impact Assessment "Revision of the Union legislation on blood, tissues and cells":  
currently evaluating the comments of the public consultation
- Submission of the legal proposal(s) by EC:  
planned for the end of 2021

# VII. Excursion- Revision of the EU- Legal Framework for Medicinal Products and Medical Devices (7) - Medical Devices Regulation

MDR: Regulation on medical devices (Regulation (EU) No 2017/745) :

- Date of Application May 26<sup>th</sup>, 2021
- so far successful implementation at least on national level
- ongoing implementation projects, e.g. EUDAMED, Common Specifications for products without a medical purpose, several guidance documents

➤ IVDR: Regulation on in vitro diagnostic medical devices (Regulation (EU) No 2017/746):

- Date of application on May 26<sup>th</sup>, 2022
- due to an incomplete regulatory infrastructure and the impact of the pandemic, there seems to be the need to extend the transitional provisions of the IVDR

# Thank you for your attention!

## Contact

German Federal Ministry of Health  
Directorate 11- Medicinal Products  
Friedrichstr. 108  
10117 Berlin

### Contact Person

Dr. Lars-Christoph Nickel  
Lars.Nickel@bmg.bund.de  
[www.bundesregierung.de](http://www.bundesregierung.de)  
Tel. +49 (0)30 18441 4600  
Fax +49 (0)30 18441-4910