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INCREASING COLLABORATION BETWEEN EU MEMBER STATES FOR PRICING AND REIMBURSEMENT

BeNeLuxA

Brussels EU Pharma Law Forum
16 May 2018

International context

- 17 June 2016: Council conclusions on Strengthening the Balance in the Pharmaceutical Systems in the EU and its Member States
  - Member States are invited to strengthen voluntary collaboration between relevant authorities and payers on pricing and reimbursement matters
  - Provide some examples of voluntary cooperation: *i.a*. 'joint horizon scanning', pro-active exchange of information; joint price negotiations in coalitions of Member States

International context II

- 2 March 2017: European Parliament resolution on EU options for improving access to medicines (2016/2057(INI))

- 16 June 2017: Council conclusions on Encouraging Member States-driven Voluntary Cooperation between Health System

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Key themes international discussions

- Ensuring patients' access to medicines
- Lack of price transparency, access to medicines endangered by very high and unsustainable price levels
- Bottom-up approach, commonly felt need to address the situation jointly
- Potential areas for voluntary structured cooperation, *i.a.*:
  - Joint horizon scanning
  - Information-sharing
  - Health Technology Assessment (HTA) cooperation
  - Voluntary price negotiations

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EU legal framework

- Fundamental right of citizens to health and medical treatment (Art. 35 Charter, Art. 8 ECHR)
- Art. 168(1) TFEU
- Free movement of goods (Art. 34 TFEU)
- Directive 89/105/EEC (“Transparency Directive”)
- Measures to prevent pharmaceutical shortages by means of a public service obligation (PSO) (Art. 81 Directive 2001/83/EC)
- Directive 2011/24/EU – application of patients’ rights in cross-border healthcare, i.a. cooperation on HTA (EUnetHTA)
- Directive 2014/24/EU – public procurement

PROPOSAL FOR A REGULATION

EU cooperation on Health Technology Assessment

About this initiative

Reference	COM(2018)51
Type	Proposal for a regulation
Full title	Proposal for a REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL on health technology assessment and amending Directive 2011/24/EU
Department	Directorate-General for Health and Food Safety

Proposal for a regulation - COM(2018)51/DOCUMENT-2018-3552

English (533 KB - PDF - 49 pages)

Download

EU Commission Proposal on Joint HTA I

- Smaller Member States more in favour (fewer resources) but concerns about higher prices and lowering of standards

- Major issues for Member States
  - Autonomy and independence of HTA agencies:
    * Autonomy: *mandatory* use of joint clinical assessment report
    * Independence: approval of joint assessments before publication
  - Availability of all evidence
  - Capacity of HTA agencies

EU Commission Proposal on Joint HTA II

- **Autonomy and independence of HTA agencies:**
  - Autonomy: mandatory use of joint clinical assessment report
    - Proposal states that "*the joint clinical assessments will be one of the main proponents of the future joint work and, following the end of the transitional period, participation in the assessments and* ***use of the joint clinical assessment reports at Member State-level will be mandatory***" and that "*Where Member States do carry out HTAs on such health technologies, there is a requirement for mandatory use of the joint clinical assessment report and* ***no repetition of the clinical assessment*** *in Member States' overall HTA processes*" (emphasis added)
    - A critical assessment of the joint clinical assessment and adaptation to the national context should always remain possible
    - HTA bodies should be able to adapt joint clinical assessment to national situation (*e.g.*, selection of appropriate comparator, use of national administrative databases, etc.)
    - Update of evidence by individual HTA body should remain possible

EU Commission Proposal on Joint HTA III

- **Autonomy and independence of HTA agencies:**
  - Independence: approval of joint assessments before publication
    - "*The proposal would place on the* ***Commission an obligation to verify the joint clinical assessment reports prior to their publication***" and "*The Commission shall publish the approved joint clinical assessment report and summary report on the IT platform*" (emphasis added)
    - HTA bodies should be able to publish their independent assessments without interference of another body
    - Commission should not have possibility to block publication of assessment
    - Authors of report should have the opportunity to respond to commission's comments
    - Researchers performing an assessment should be allowed to publish their findings on website of their HTA institute and/or in peer-reviewed journals free from outside interference

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EU Commission Proposal on Joint HTA IV

- **Availability of all evidence**
  - Proposal states that "*the designated sub-group shall* ***request the health technology developer to submit*** *the documentation containing the information,* ***data and evidence*** *necessary for the joint scientific consultation*" (emphasis added)
  - HTA experts are confronted with a major problem of publication and reporting bias.
  - The proposal includes insufficient obligations for the technology developers to provide all evidence:

    1) Timely prospective registration of all trials:

    This should allow assessors to check whether all evidence has been submitted. The timely registration should be monitored and necessary steps should be taken if the technology developer fails to comply.

    2) Provide a full list of all studies:

    A list should be provided of all studies in which the technology has been used. The status of these studies should be provided (ongoing, stopped, finished, etc.). The results or reasons for stopping the study should be provided.

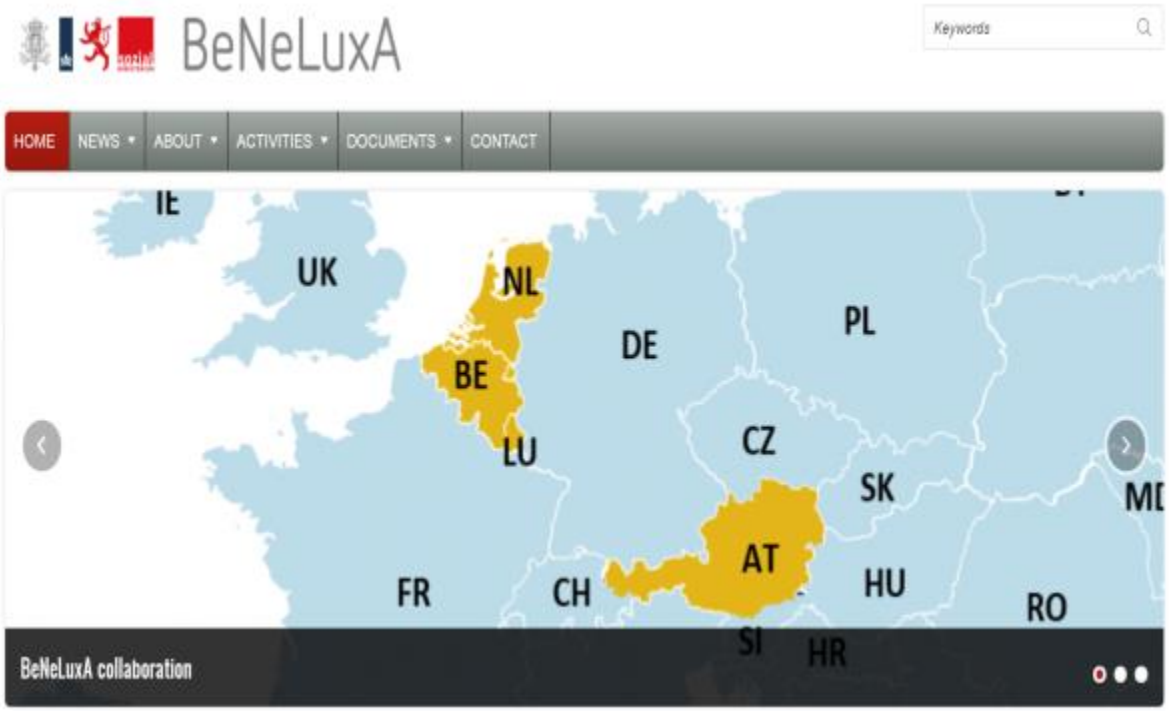
    3) Information should be provided in a transparent and structured manner:

    The technology developers should submit their data according to a standardised template (e.g. ordered per study type, proper summary tables (e.g. on adverse events), access to underlying data to be able to check the information in the file, etc.).

EU Commission Proposal on Joint HTA V

- **Capacity of HTA agencies**
  - Proposal states that "***Participation*** *in the assessments and use of the joint clinical assessment reports at Member State level* ***will be mandatory***", that "*The designated authorities and bodies should ensure an appropriately high level of representation in the Coordination Group and technical expertise in its sub-groups, taking into account the need to provide expertise on the HTA of medicinal products and medical devices*", that "*Following the end of the transitional period,* ***all medicinal products falling within the scope and granted marketing authorisation in a given year will be assessed****, while a selection of medical devices falling within the scope will undergo assessment*" and that "*Members States which are already participating should not be allowed to withdraw from the framework for joint work*" (emphasis added)
  - It is very questionable that all HTA agencies have sufficient capacity to perform their work for both their own government as well as the work for all medicinal products falling within the scope.

BeNeLuxA


BeNeLuxA
Keywords
HOME
NEWS
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IE
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BeNeLuxA collaboration

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Participants

- Initiated by Belgium and the Netherlands (April 2015)
- Later joined by Luxembourg (September 2015) and Austria (June 2016)
- Population covered by initiative is 37 million inhabitants

- *Note: Other initiatives exist, e.g. Valetta Declaration group, Visegrad group and Nordics cooperation, but at present are less advanced*

Future participants?

- BeNeLuxA is willing to consider including other countries (including non-EU) into their proposal
- Ireland and Switzerland* are interested in joining the initiative; status Ireland: letter of intent
- France* is interested in collaborating with the group on (1) Exchange of information; (2) Horizon Scanning; and (3) HTA
- Italy* has also approached the Netherlands to explore the possibilities for further collaboration (informally)

* Sources: Dutch Parliamentary documents (*Kamerstukken II* 2016/17, 29477, 414, p. 17, 31-32, 34, 46, 49; *Kamerstukken II* 2016/17, 29477, 419, p. 12, 19; *Kamerstukken II* 2016/17, 21501-13, 446)

Transparency?

- Work in progress
- Only little information available on nature, scope, criteria and practical details of the BeNeLuxA Initiative
- Scattered information
- Government-run website with public information re. initiative: www.beneluxa.org

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Legal context

- Constitutional systems countries
- EU law (i.a. free movement of goods, Transparency Directive, Directive 2011/24/EU)
- BeNeLuxA is not based on a treaty
- Based on letters of intent and additional working agreements for administrative consultations, *i.a*. joint horizon scanning agreement (BeNeLuxA – Terms of Reference)

Scope

- ‘BeNeLuxA’ intends to collaborate more closely across a range of areas
- Initiative goes beyond jointly negotiating with the pharmaceutical industry
- Initiative is not limited to orphan drugs, could also concern other products and product combinations (*e.g*. combinations of medicines)

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Main objectives

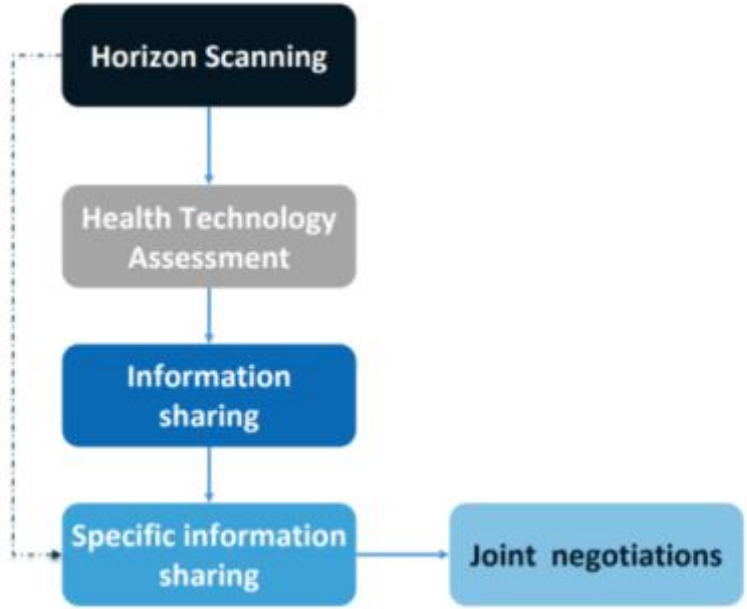
1. Horizon Scanning
2. Exchange of information
3. Health Technology Assessment
4. Joint negotiations on pricing

Collaborative approach to gathering information

- Mutual recognition
- Joint HTA

- Best practices
- Registries
- Policy dilemmas

- Horizon scan interpretation
- Strategic information exchange
- Input for joint negotiations



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Main objectives

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Objectives of the collaborative horizon scanning system (HSS)

- Informing decision-makers on emerging and new medicines for reimbursement decisions and policy development
- Informing decision-makers on issues that are relevant for the managed introduction and monitoring of medicines
- Facilitate budget impact estimation and budget planning
- Product selection for (international collaboration on) early dialogue with industry, price negotiations, HTA and registries
- Planning health services

Scope

- “Both inpatient and outpatient pharmaceutical products with a potentially high financial, clinical and/or organizational impact on the health system”
- In scope: first biosimilar for a biological, ATMPs
- Out of scope (for the time being): prophylactic vaccines, generics and medical devices

Two types of Horizon Scanning

1. Joint Horizon Scanning by Belgium, The Netherlands, Luxembourg and Austria

2. International Joint Horizon Scanning – a separate initiative by Belgian Minister of Public Health
   - Based on model developed by Belgian Health Care Knowledge Center
   - Expected to be operational in 2018 or 2019
   - Aim: involve as many countries as possible to increase knowledge of drugmakers' pipelines around the world.
   - Interested countries currently include *inter alia* Belgium, Sweden, Iceland, Canada, Poland and Switzerland
   - Discussions ongoing on sharing of costs
   - System should help BeNeLuxA countries to identify new medicines for joint pricing negotiations.

   - *Note: This does not require full participation in the BeNeLuxA Initiative*

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Points to consider

- Input from company (or other parties) to Country A can influence processes in Countries B, C, D, etc.
- Database could include confidential and sensitive information from/about companies as well as from EMA
- Commercially confidential information and other sensitive information should be kept confidential
- Central horizontal scanning unit could be (part of) a national governmental agency
- Applicability FOIA rules of country/countries involved (major issue under Dutch law)

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Main objectives

1. Horizon Scanning
2. **Exchange of information**
3. Health Technology Assessment
4. Joint negotiations on pricing

Exchange of information

- Proactive exchange of information on pharmaceutical markets, prices, “best practices”, experience and disease-specific cross-border registries

- Collaborating BeNeLuxA countries intend to be transparent and share prices that will be or even have been negotiated on a national level (insofar as financial agreements with pharmaceutical companies would allow them to do so)

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Main challenges

- Exchange of confidential and sensitive information from/about companies
- Companies that have negotiated or will be negotiating financial arrangements with governmental agencies need to be aware of the BeNeLuxA goal to share information (carefully check confidentiality clauses in contract)
- Commercially confidential information and other sensitive information should be kept confidential
- Enforcement?

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Main objectives

1. Horizon Scanning
2. Exchange of information
3. **Health Technology Assessment**
4. **Joint negotiations on pricing**

Joint Health Technology Assessment (HTA) & Joint negotiations on pricing

- No public procurement or actual purchasing of medicines

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Framework joint assessment and joint negotiations

- Still a pilot phase
- Scope: not limited to orphan medicines; open to any medicine with a significant budgetary or therapeutic impact. Currently no plans to include medical devices
- Frameworks for joint assessment and joint negotiations are still under development at BeNeLuxA level
- Aim is to give insight into the procedures and requirements as soon as possible
- At EU level: Proposal for a Regulation of the European Parliament and of the Council on health technology assessment and amending Directive 2011/24/EU (31 January 2018)

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Four types of HTA collaboration

- **Re-use of HTA reports**
  Countries use parts of HTA-reports of other countries
- **Joint HTA report**
  Authors of several countries join forces in order to write one report, which can then be used in all the countries involved
- **Mutual recognition**
  HTA-report of one country (in part or full report) is adopted by others in a parallel process; the results of the assessments are then published at the same time
- **External referee**
  HTA institutes of the various countries act as an external referee for another country in national procedures. It does not involve active work in HTA itself

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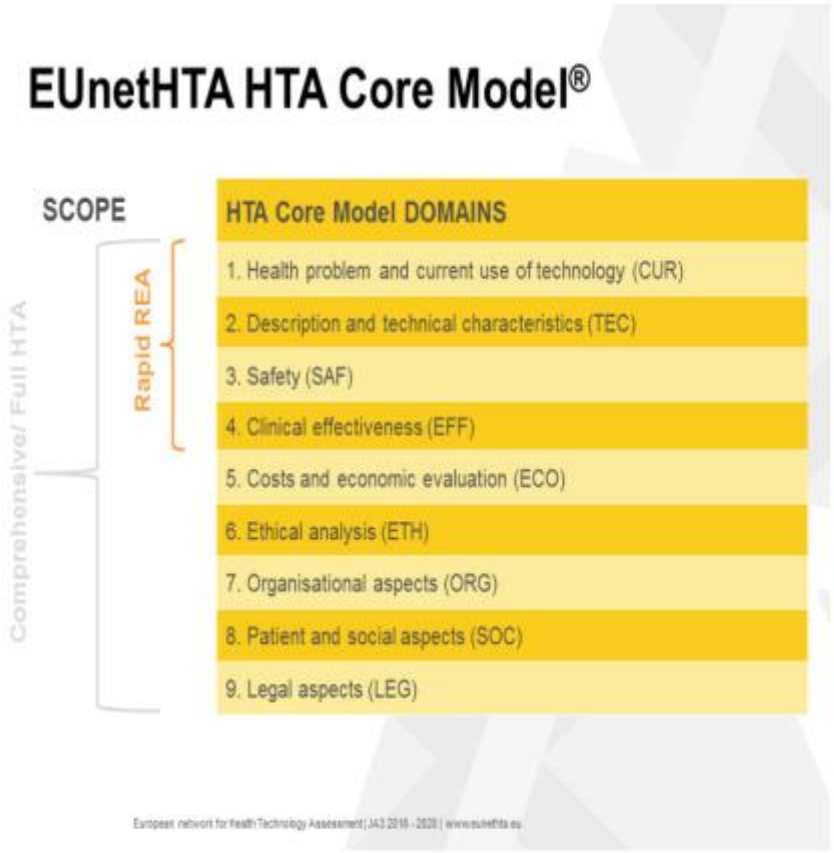
First results of joint HTA procedures (October 2017)

Name pharmaceutical	active substance (EMA)	therapeutic area (EMA)	year	Type of HTA-collaboration
LOJUXTA	lomitapide	hyper-cholesterolemia	2015	Re-use of Dutch work by Belgium
ORKAMBI	lumacaftor / ivacaftor	cystic fibrosis	2016 first submission	Joint writing by Belgium & The Netherlands The Dutch Zorginstituut also acted as external referee for RIZIV-INAMI Final report was used by Luxembourg
PRALUENT	alirocumab	dyslipidemias	2016	Dutch Zorginstituut acted as external referee for Belgium RIZIV-INAMI
ORKAMBI	lumacaftor / ivacaftor	cystic fibrosis	2017 second submission	Joint writing by Belgium & The Netherlands The Dutch Zorginstituut also acted as external referee for RIZIV-INAMI Final report was sent to Luxembourg and Austria
VYNDAQEL	tafamidis	amyloidosis	2017	The Dutch Zorginstituut acted as external referee for RIZIV-INAMI Final report was used by Luxembourg

The Table mentions the situation in October 2017.

Abbreviations: RIZIV-INAMI Rijksinstituut voor Ziekte- en Invaliditeitsverzekering Institut National Assurance Maladie-Invalidité(Belgian HTA activities on submitted pharmaceutical files for reimbursement); EMA European Medicines Agency

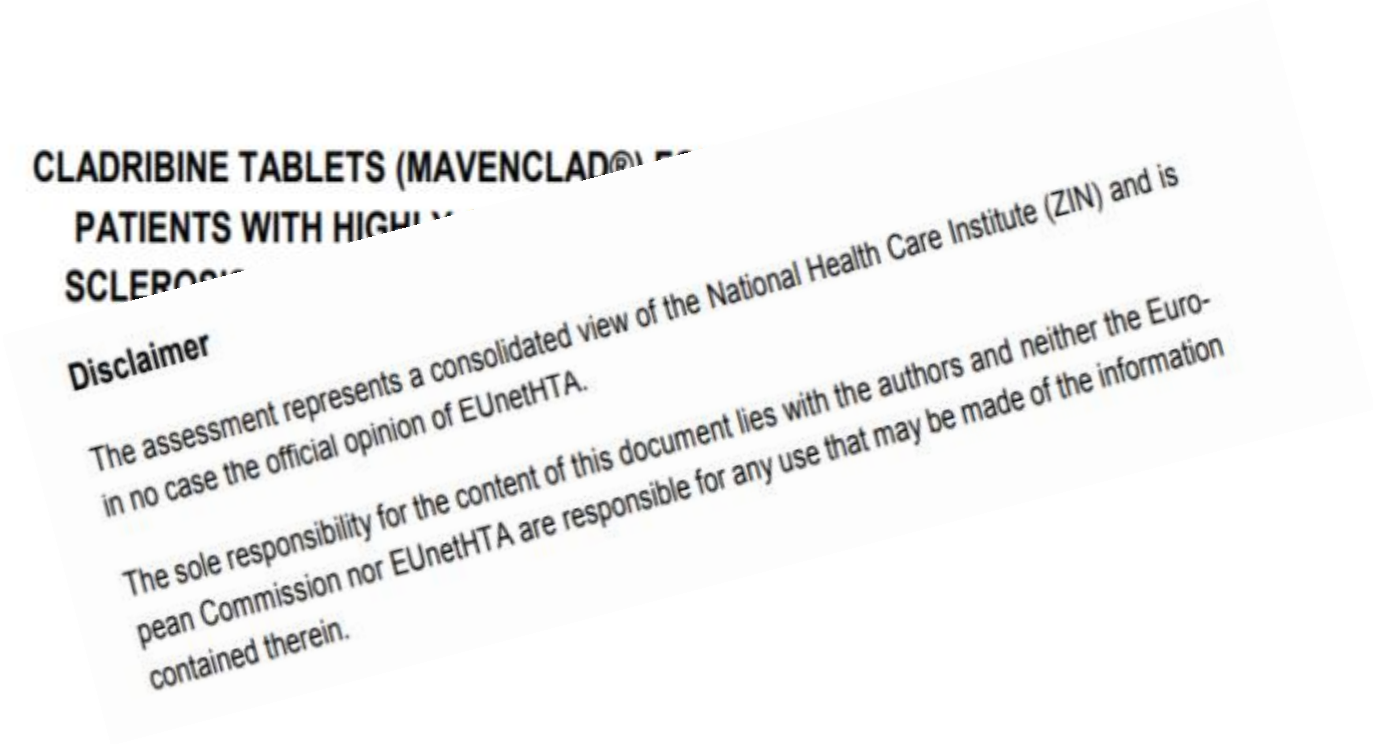
The HTA Core Model® is a *methodological framework* for production and sharing of HTA information. The HTA Core Model® is a registered trademark.



The model consists of the following three components, each with a specific purpose:

Zorginstituut Nederland

Pharmacotherapeutic assessment using the rapid relative effectiveness assessment format



CLADRIBINE TABLETS (MAVENCLAD®)

PATIENTS WITH HIGH

SCLERO

Disclaimer

The assessment represents a consolidated view of the National Health Care Institute (ZIN) and is in no case the official opinion of EUnetHTA.

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Potential benefits

- Potential significant positive impact for patients
- Could expedite access to product in countries involved
- Reduce workload for company and countries involved: only one dossier, one joint negotiation, single outcome for all the countries involved
- Workload of national agencies → pilot / joint procedure priority

- BUT: all joint pricing negotiation pilots have failed so far (e.g. ORKAMBI® – Vertex)

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Process joint assessment and joint negotiations

- Kick-off meeting
- Framework for joint assessment and negotiation procedures
- If outcome of joint HTA is positive, subsequent joint negotiations
- Joint assessment can be done independently of joint negotiations

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Process joint assessment and joint negotiations II

- Separate reimbursement procedures for countries involved, make sure framework allows for a continuation of national procedures if joint assessment/negotiations are discontinued, milestones
- In case of successful joint assessment and negotiations, reimbursement decisions will be made simultaneously in countries involved
- Outcome joint negotiations set out in specific terms in each country in separate (confidential) national Financial Arrangements

Main challenges

- Starting points for the pharmaco-therapeutic assessment have to be similar in the countries, any differences in national clinical practice, criteria, etc. have to be identified beforehand (*e.g*. different start criteria for treatment)
- Countries use different application templates
- Pharmaco-economic evaluation required for orphan drug?
- Rationale for joint negotiations if product is cost-effective?
- Projected timelines, including clock-stops, face-to-face meetings, oral hearings → will the process be derogatory to the standard processes and timelines (e.g. Austria: shorter timelines for producing assessment reports)? Apparently *“too early to consider full procedural harmonisation”*
- Do the national rules allow consultations of experts in the field?

Main challenges II

- Confidentiality of exchanged price data (applicability FOIA rules of country/countries involved)?
- Language of communications: Dutch, French, German, English? (*e.g*. no legal requirement in the Netherlands to draft agreements in Dutch; Belgium and Austria: not possible to submit HTA application in English and/or to draft authorities' assessment report in English; etc.)
- Governing law and jurisdiction?

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EU law perspective - discussion

- Directive 89/105/EEC?
  - De facto price control (Art. 2)?
  - Mechanism for positive/negative list (Art. 6/7)?
  - Direct or indirect control on profitability (Art. 5)?
- Transparent, objective and verifiable criteria?
- Adequate legal remedies?

Collaborative approach to gathering information

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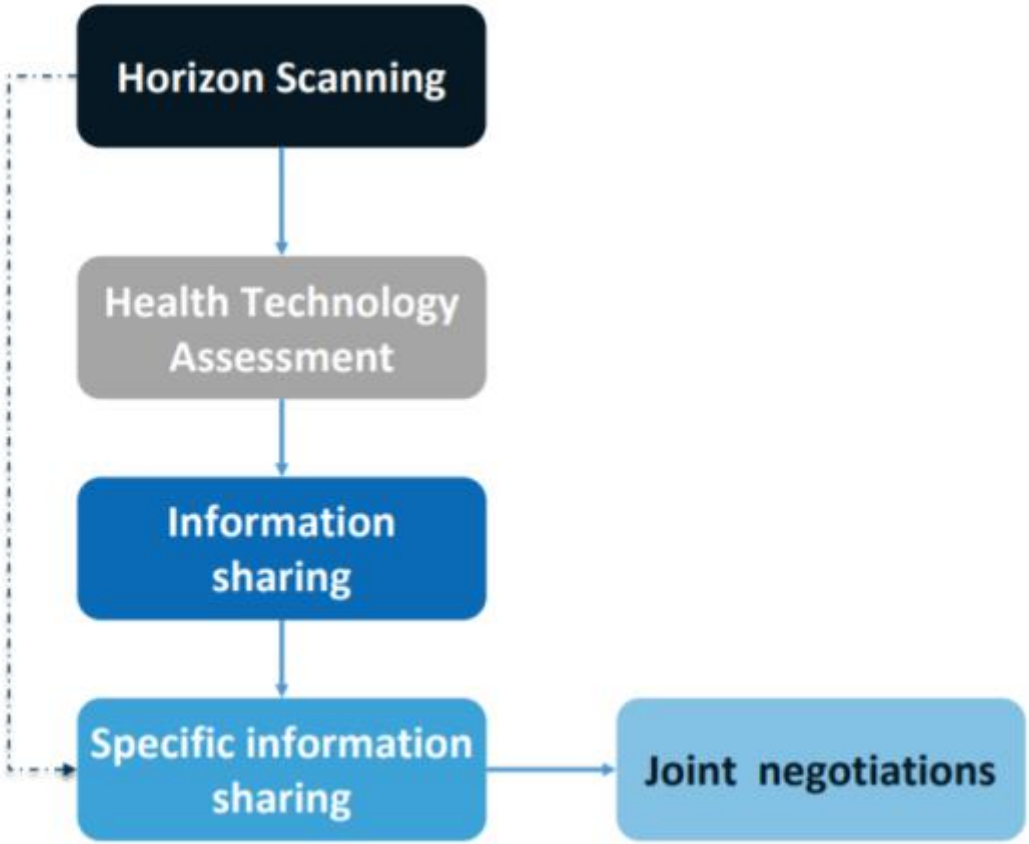

Austrian EU presidency (July – December 2018)

- Focus on "Ensuring access to medicines" during Austrian EU presidency in second half of 2018
- Access to innovative medicines vs sustainable financing of health systems
- BeNeLuxA is seen as a way to accomplish this goal
- Austria most interested in exchange of information; HTA assessment analysis; price negotiations
- Horizon scanning is seen as being of less importance for Austria

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Any questions??

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