HAUTE AUTORITÉ DE SANTÉ

HTA for Orphan Drugs in France and European Cooperation

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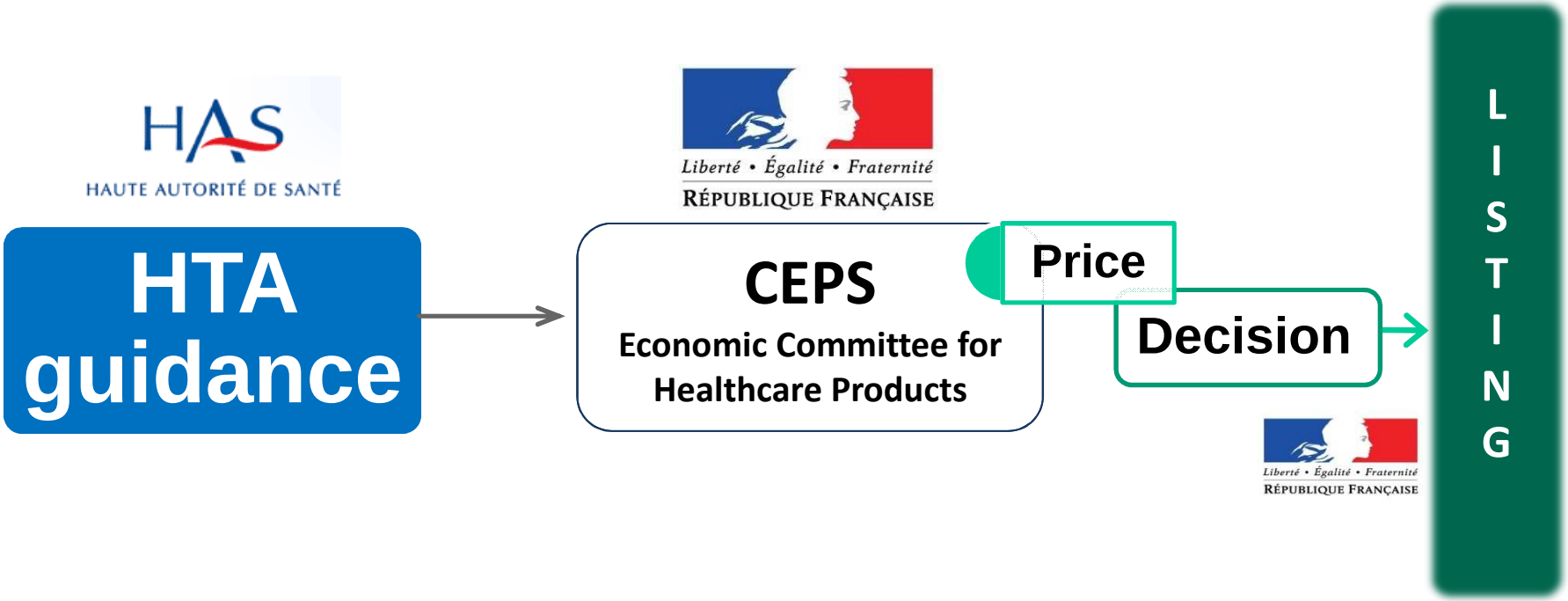
Advisor to the President
HAS

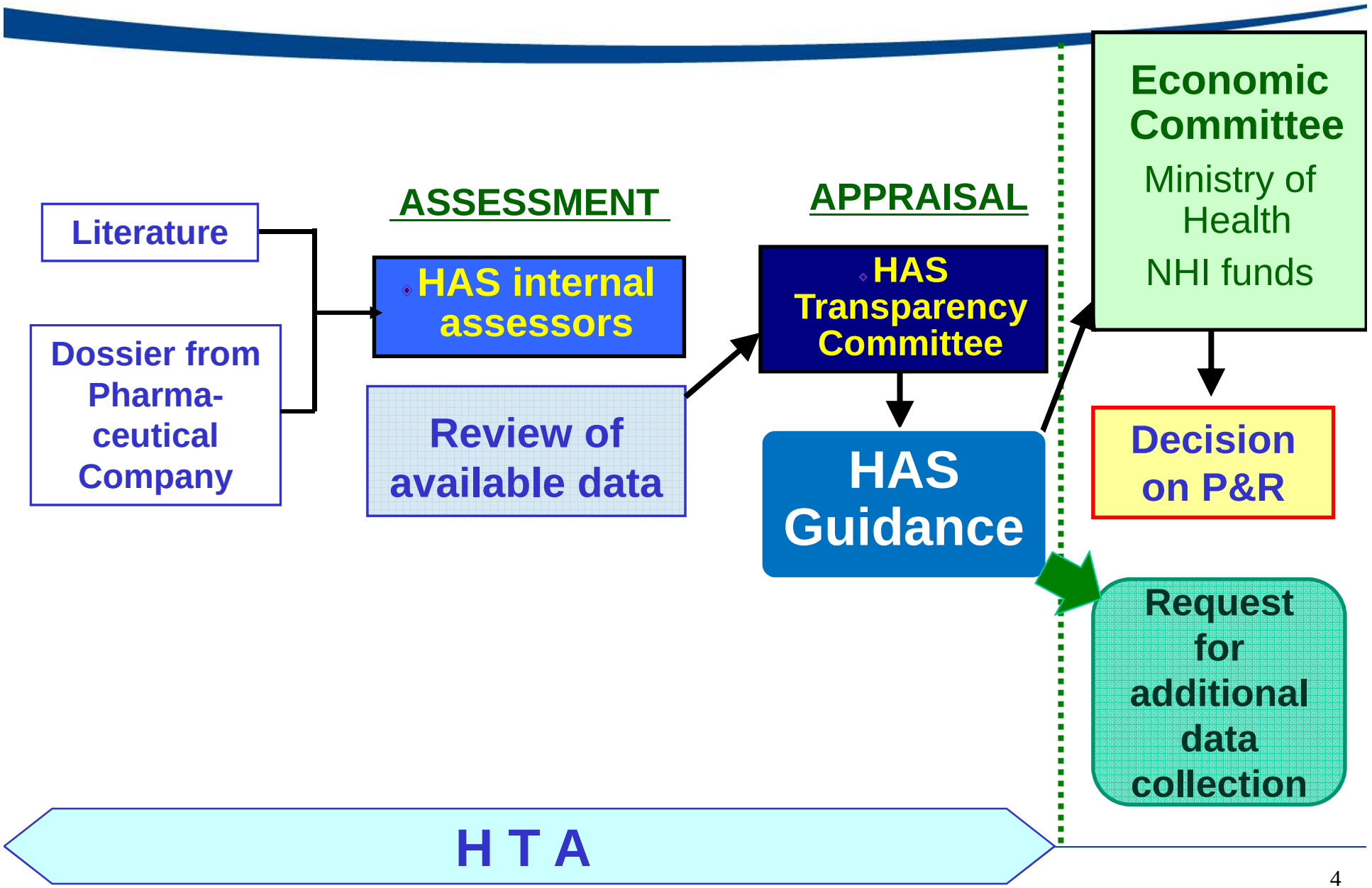
01

HTA and reimbursement in France and in Europe

HTA, Reimbursement and Pricing for a new drug: The main actors

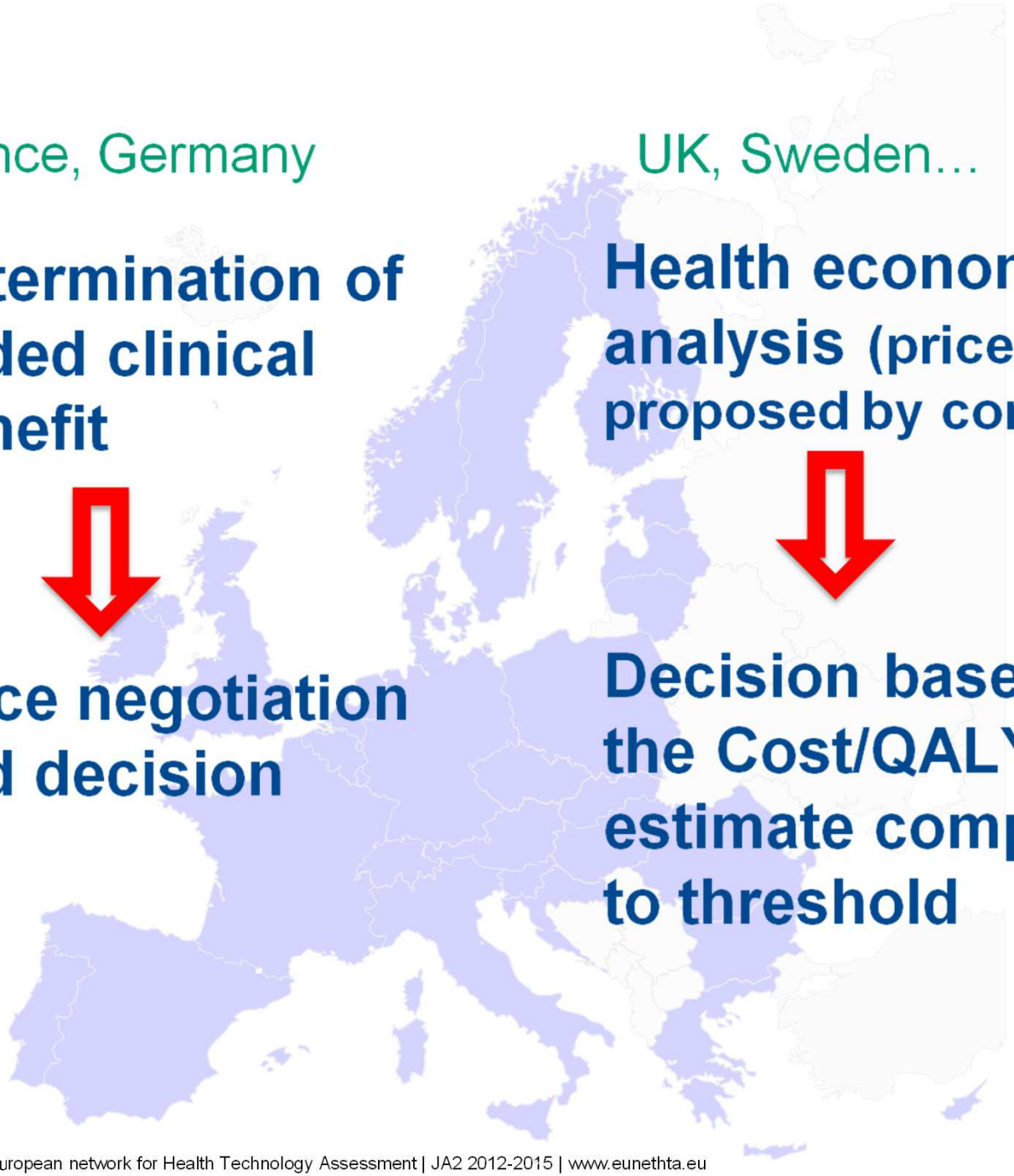


From HTA to pricing and reimbursement



Added Clinical benefit (ASMR) and Price

Added clinical benefit	ASMR	Price
Major Important Moderate	I II III	Higher Price than comparators. « European » price accepted.
Minor	IV	No important difference in price / comparator
No Added Clinical benefit	V	Reimbursement ONLY if price inferior to comparators



France, Germany

Determination of added clinical benefit

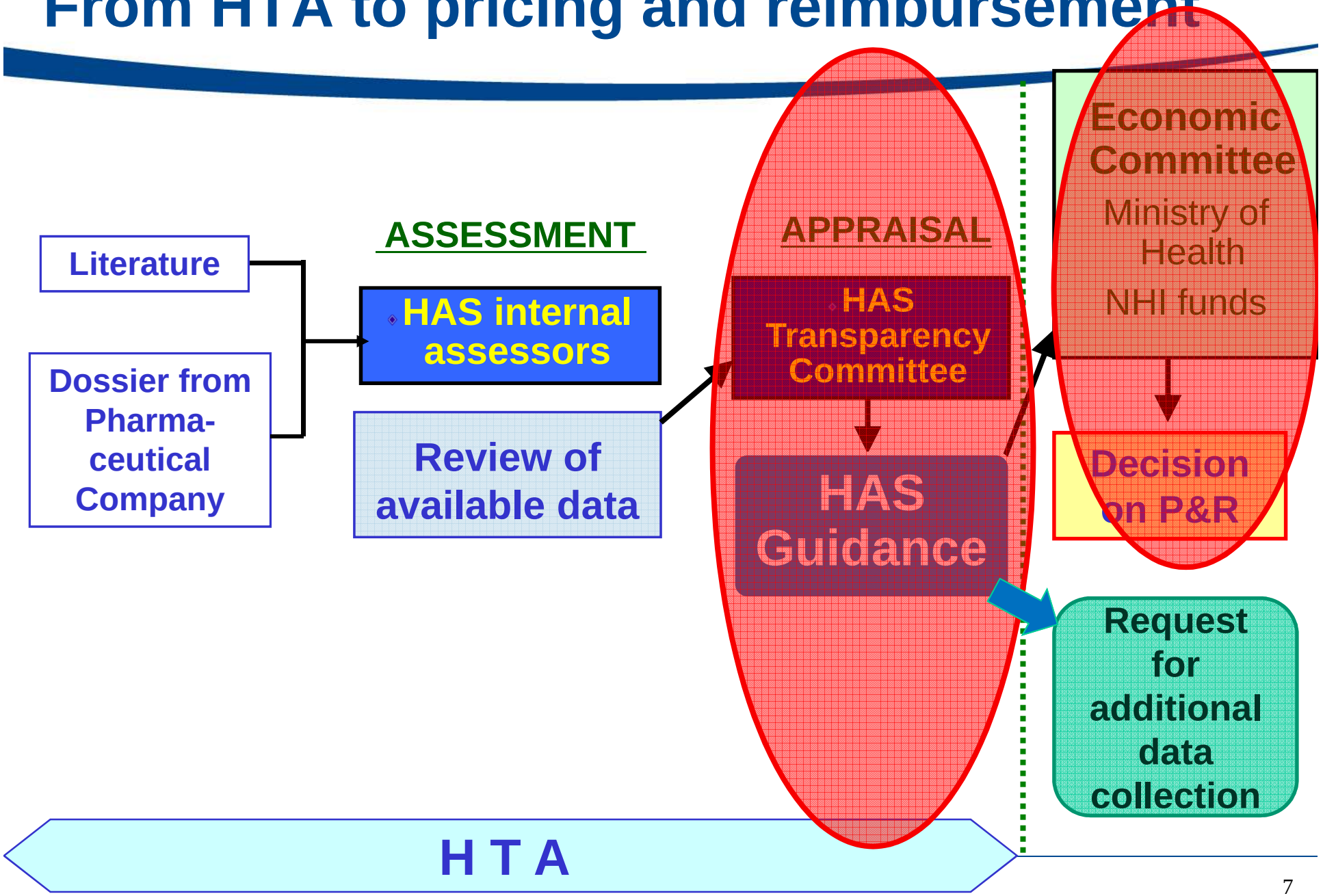
Price negotiation and decision

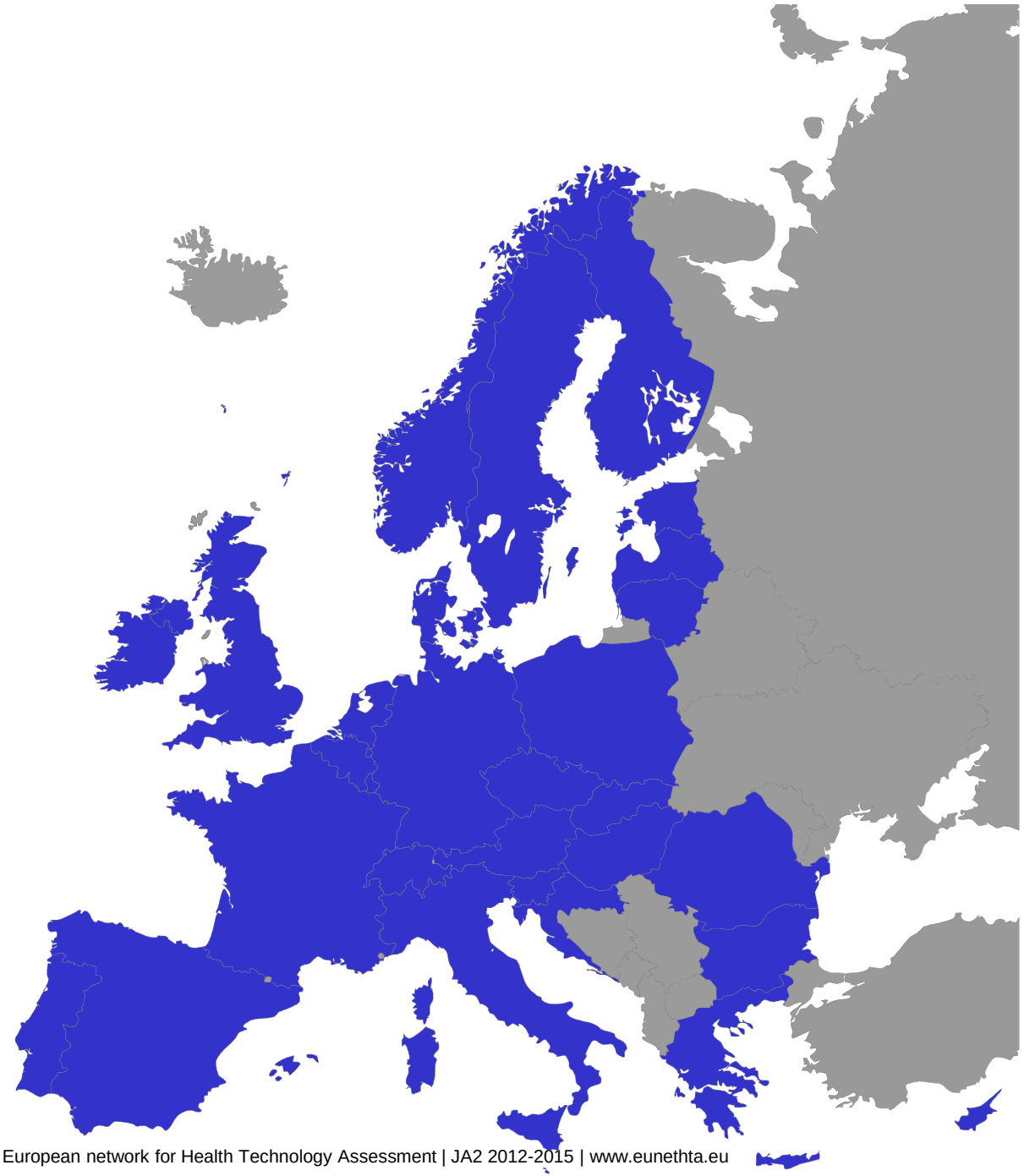
UK, Sweden…

Health economics analysis (price proposed by company)

Decision based on the Cost/QALY estimate compared to threshold

From HTA to pricing and reimbursement





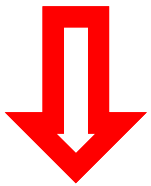
02

Ongoing changes in France

Evolution of the French system

Determination of added clinical benefit

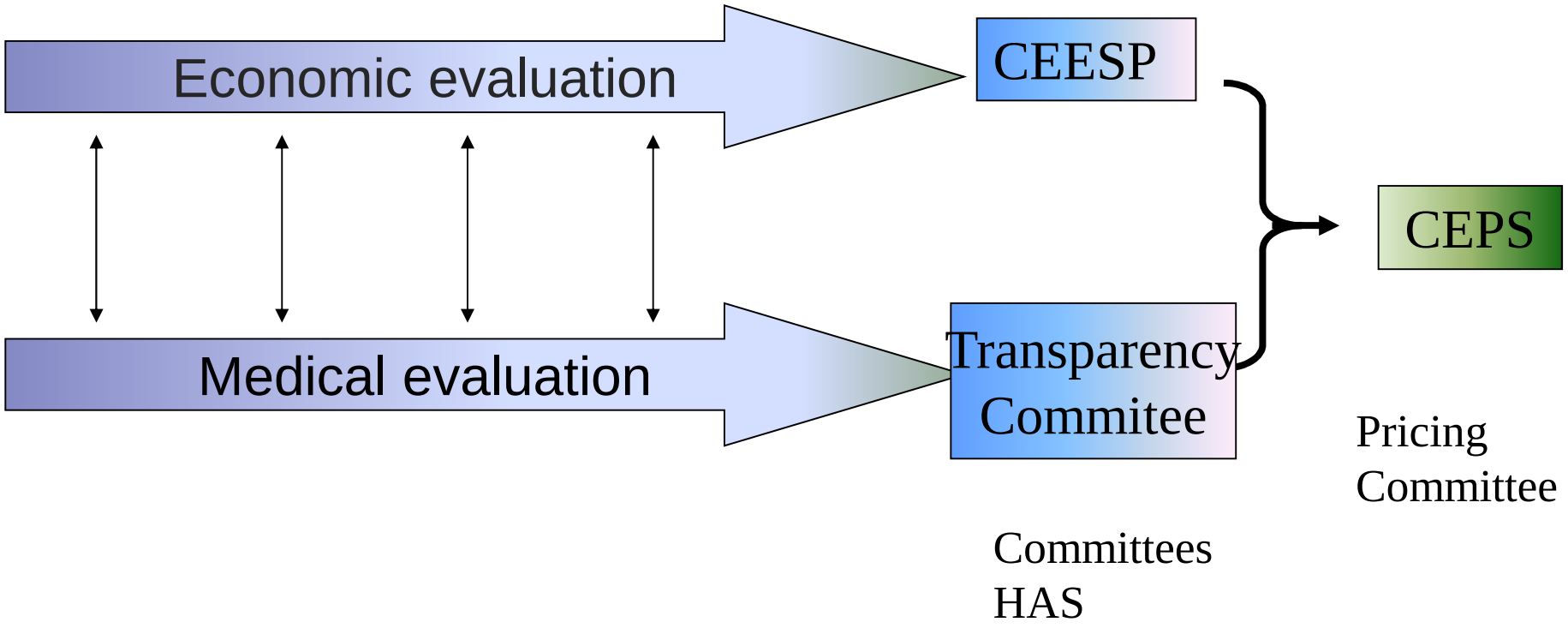
Towards a new criterion?



Price negotiation and decision

Introduction of economic evaluation to support decision on price

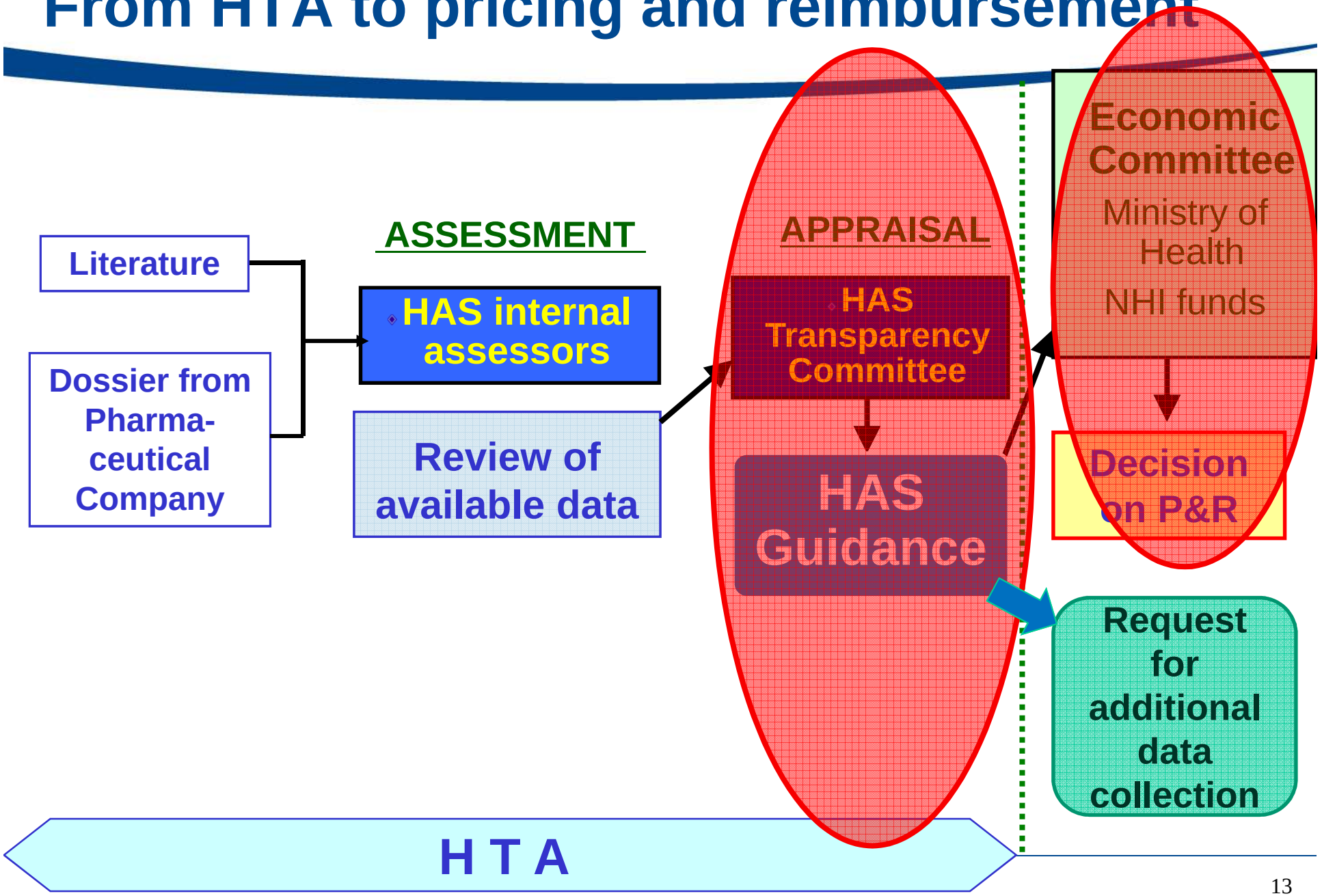
Instruction coordonnée, deux avis remis au CEPS



03

HTA cooperation in Europe is progressing fast!

From HTA to pricing and reimbursement



Should HTA bodies work separately or can we share common Assessments?

- **EUnetHTA pilots on Rapid Assessments**
- **Coordinated by CVZ (Work Package 5)**

Core HTA information for rapid assessment of drugs and devices
Health problem and current use of technology
Description and technical characteristics of the technology
Safety
Effectiveness

More pilots needed. Companies advised to volunteer !

For assessing drugs, can we adopt common methodological standards?

- **Assessment methods: EUnetHTA Methodological guidelines**

  ► Already produced : 9 guidelines for the assessment of drugs

  Available on EUnetHTA website!

  - Choice of comparator
  - Clinical endpoints
  - Composite endpoints
  - Surrogate endpoints
  - Direct and indirect comparisons
  - HRQoL
  - Safety
  - Internal validity
  - Applicablity

  ► To be produced: Guidelines for the assessment of medical devices and procedures

  Lead : HAS, Co-Lead: IQWIG

Can we improve the appropriateness of data?

- **Early Dialogue between HTA bodies and companies**
  - Scientific advice (SA) in place for a long time at regulatory agencies ,
  - Some HTA bodies implemented national SA activities
- **Early dialogue / Scientific advice**
  - Multi HTA early dialogue (EUnetHTA)
  - EMA HTA early dialogues (EMA)

Appropriateness of data (2)

Disease oriented approach: Disease specific guidelines

- EUnetHTA project. Lead: HAS
  1 or 2 Disease specific guidelines
- First condition chosen: Osteoarthritis
  - All stakeholders were given the possibility to make proposals
  - Concept paper disseminated for comments then published on EUnetHTA website (Main author Osteba, Spain)

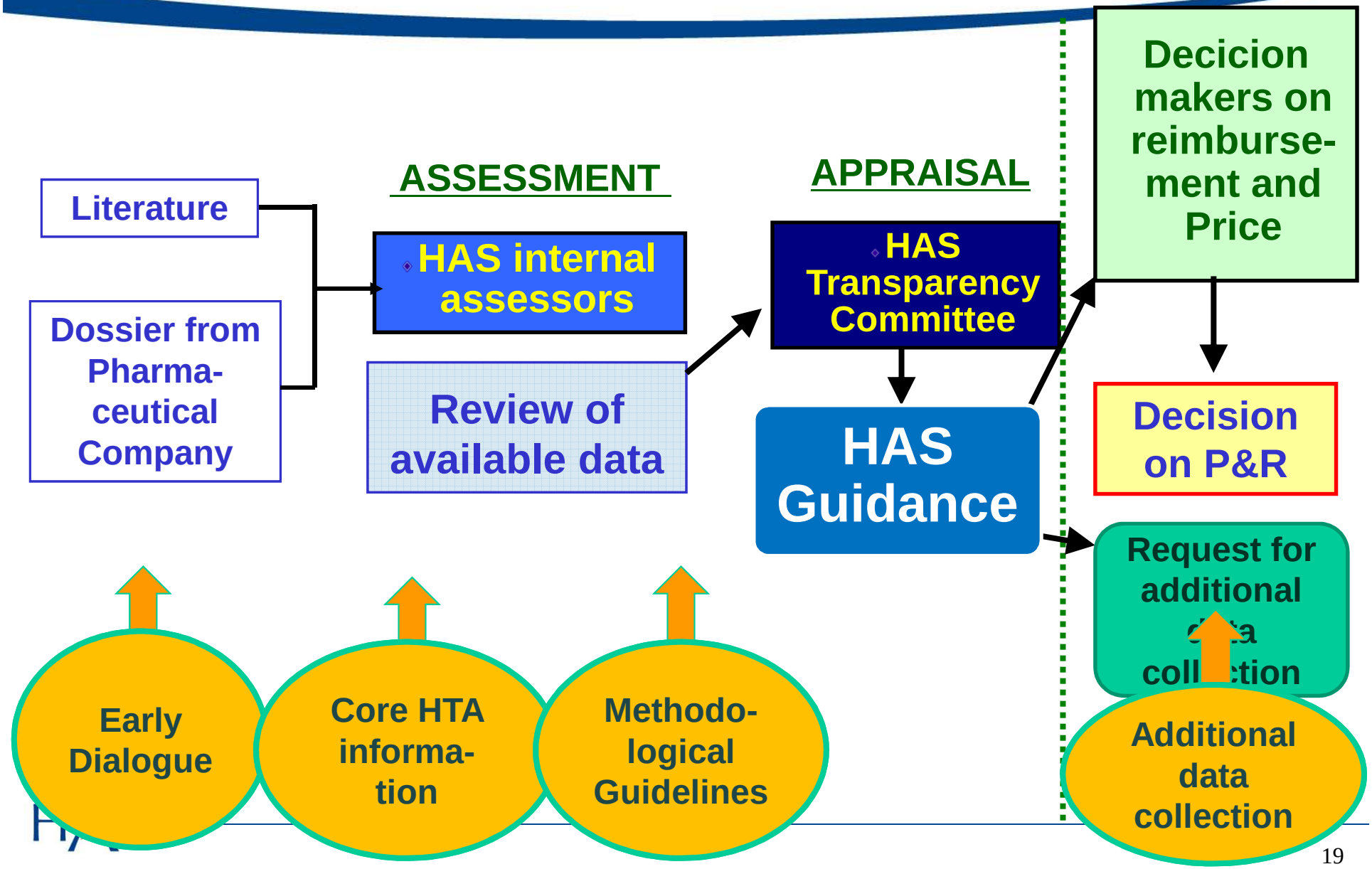
Appropriateness of data (3)

Additional data collection: EUnetHTA objectives

- Definition of **criteria** to select new technologies in need of further evidence (published)
- **database** (EVIDENT) to share information & facilitate collaboration on additional evidence generation (launched Nov. 5th, 2012)
- For some technologies, cooperation of several HTA bodies to define a common research question and a « **common core protocol** »
- EMA – EUnetHTA cooperation
- Cooperation with ENCePP European network of centers of pharmaco epidemiology

Soon to come: Chose of pilots (Drug, device, other..)

Areas for cooperation / harmonisation



EMA Workshop on Early Dialogues

26 Nov 2013

Breakout session 4

Special Areas: Orphan drugs / ATMPs / Paediatrics / Personalized medicines / Vaccines

Moderators

- **Regulatory:** Bertil Jonsson
- **HTA:** François Meyer
- **Industry:** Thibaut du Fayet

Main issues

- **Target population indication specificity** (Orphan, Paediatrics, Personalized medicine): **limited and/or specific population**
- **Complex therapeutic interventions (ATMPs, Orphan, Paediatrics, Personalized medicine): high product complexity with new MoA, often in « niche » diseases, with increasing co-developments (Rx/DX)**
- **Development & Market access burden (Orphan, ATMPs, Paediatrics, Personalized medicine): level of requirements similar to standard products (assesment process & guidelines)**
- **Companies lack of expertise (Orphan, ATMPs, Paediatrics): Innovative SMEs, often positioned in these particular situations, with limited internal expertise & dedicated resources**

Potential solutions

Category	Proposals
Scientific	• Anticipate prospective meta analysis • Data on non-clinical models • Pharmaceutical paediatrics formulations
Process	• Early HTA value assessment (non binding) within the scientific advice • Methodological guidelines alignment between HTA / EMA • EMA / HTA parallel Qualification advice for novel methodologies • HTA to attend EMA working parties / committees? • Share of expertise network, managing conflict of interest • Make use of the EU experts network (meta network Enpr-EMA, EUCERD, Paediatrics) • Reassessment of Long Term follow-up to be discussed during early parallel advice (ATMPs)
Policy	• Make transparent contribution to advice from COMP and PDCO • Disease Guidelines production, based upon EMA existing guidelines • Patients involvement • Organizational challenges (ATMPs)

The different pathways existing for multi HTA early dialogues

EMA - HTA Scientific Advice

- HTA bodies chosen by companies
- Exchange between agencies: during pre-meeting teleconference (involving EMA and HTA bodies) ; not on the day of the meeting
- No organised share of conclusions
- Fees for companies for EMA, certain HTA bodies
- EunetHTA observer?

EUnetHTA Early Dialogues:

- Part of EU Health programme 2008-2013
- Actors are voluntary HTA bodies, national or regional
- EU objectives to be taken into account
  - Capacity building
  - Inter agencies discussion on key issues
- Learning curve:
  - Training provided
- EMA as an observer
- No fees for companies

NEW

Additional EDs (2014)
EC Call for tender

- In addition to EunetHTA ED
  - **At least 10 EDs - 7 drugs and 3 MD/diagnostics/procedures**
  - **At least 10 HTA organisations.**
- Consortium HAS (lead) + 13 partners, selected by EC
- ***SEED: Shaping European Early Dialogues***
- Regulators, payers, patient representatives as observers.
- Sustainable process to put in place, including collaboration with EMA
- Kick off meeting (D1): October 21, 2013
- Preliminary work : procedures and templates for
- All ED in 2014, interim report aft
- Scenarios to test:
  - **Sequential/independe**
  - **Parallel EMA-HTA advi**
- ***Model for permanent network at the end***

Call for expression of interest to be published soon !!!

HAS

Conclusion

- P&R decision processes and criteria for appraisal are still, and will remain, country specific
- Voluntary cooperation between HTA bodies received a political support and is more and more a reality
- Significant actions are possible within this network to reduce unjustified differences between local HTA reports/guidance and to improve the quality of the data produced by technologies sponsors
- Political and regulatory limits in convergence does not limit Scientific exchanges and cooperation

Thanks for your attention

EUnetHTA

European network for
Health Technology Assessment