Relative effectiveness assessment of pharmaceuticals (WP5) EUnetHTA JA1

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Background

Pharmaceutical Forum 2008 Recommendations

- Decisions on reimbursement on national level
- Relative effectiveness assessment (REA) vs cost-effectiveness assessment (CEA)
- Exchange of REA criteria/information
- Implementation of agreed good practice principles for REA
- More effectively done by existing networks

- EUnetHTA was asked to take this work forward by the Steering Committee of the HL PF in autumn of 2008.
- EUnetHTA decided to work with the definitions that had been agreed in PF2008
- EUnetHTA WP on REA started in 2010

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Working definition

According to the Pharmaceutical Forum:

- **Relative effectiveness** can be defined as the extent to which an intervention does more good than harm compared to one or more intervention alternatives for achieving the desired results when provided under the usual circumstances of health care practice.

The WP5 Partners

1 Lead

College voor zorgverzekeringen

1 Co-lead

HAUTE AUTORITÉ DE SANTÉ

17 Associated Partners

National Institute for Health and Clinical Excellence

kunnskapssenteret

Syddansk Universitet

KCE

National Institute for Strategic Health Research

IQWiG Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen

Ministerstvo zdravotnictví České republiky

National Institute of Public Health

Gesundheit Österreich GmbH

Health Information and Quality Authority

infarmed Autoridade Nacional do Medicamento e Produtos de Saúde I.P.

gov.mt

Agenzia Italiana del Farmaco AIFA

National Institute for Health and Welfare

Veselības ekonomikas centrs

12 Collaborative Partners

Generalitat de Catalunya www.gencat.cat

Comunidad de Madrid

La Suma de Todos

RegioneLombardia

RIZIV

TLV

Università Cattolica del Sacro Cuore

IRF Institut for Rationel Farmakoterapi

Kanita Dayalı Tıp Derneği kdtd Turkish Evidence Based Medicine Association

jazmp Agency for Medicinal Products and Medical Devices of the Republic of Slovenia

AETSA

SNHTA

EUnetHTA JA1 WP5 REA Pharmaceuticals

- **Background review on national REA (CVZ)**
- **Methodology guidelines for REA (HAS)**
- **Development of (rapid) REA model (CVZ)**
  - Including pilot on REA model
- **Collaboration with EMA (CVZ and HAS)**

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Background review on national REA

Completed	No information
29 countries:	***5 countries***
Austria	**Cyprus**
Hungary	**Greece**
Latvia	**Lithuania**
New Zealand	**Romania**
Poland	**Bulgaria**
Luxembourg	
France	
Switzerland	
Ireland	
United Kingdom*	
Canada	
Norway	
Sweden	
Finland	
Spain	
Slovenia	
Netherlands	
Germany	
Belgium	
Estonia	
Czech Republic	
Australia	
Slovakia	
Portugal	
USA	
Turkey	
Italy	
Malta	
Denmark	

***Separate data abstraction for England/Wales & Scotland**

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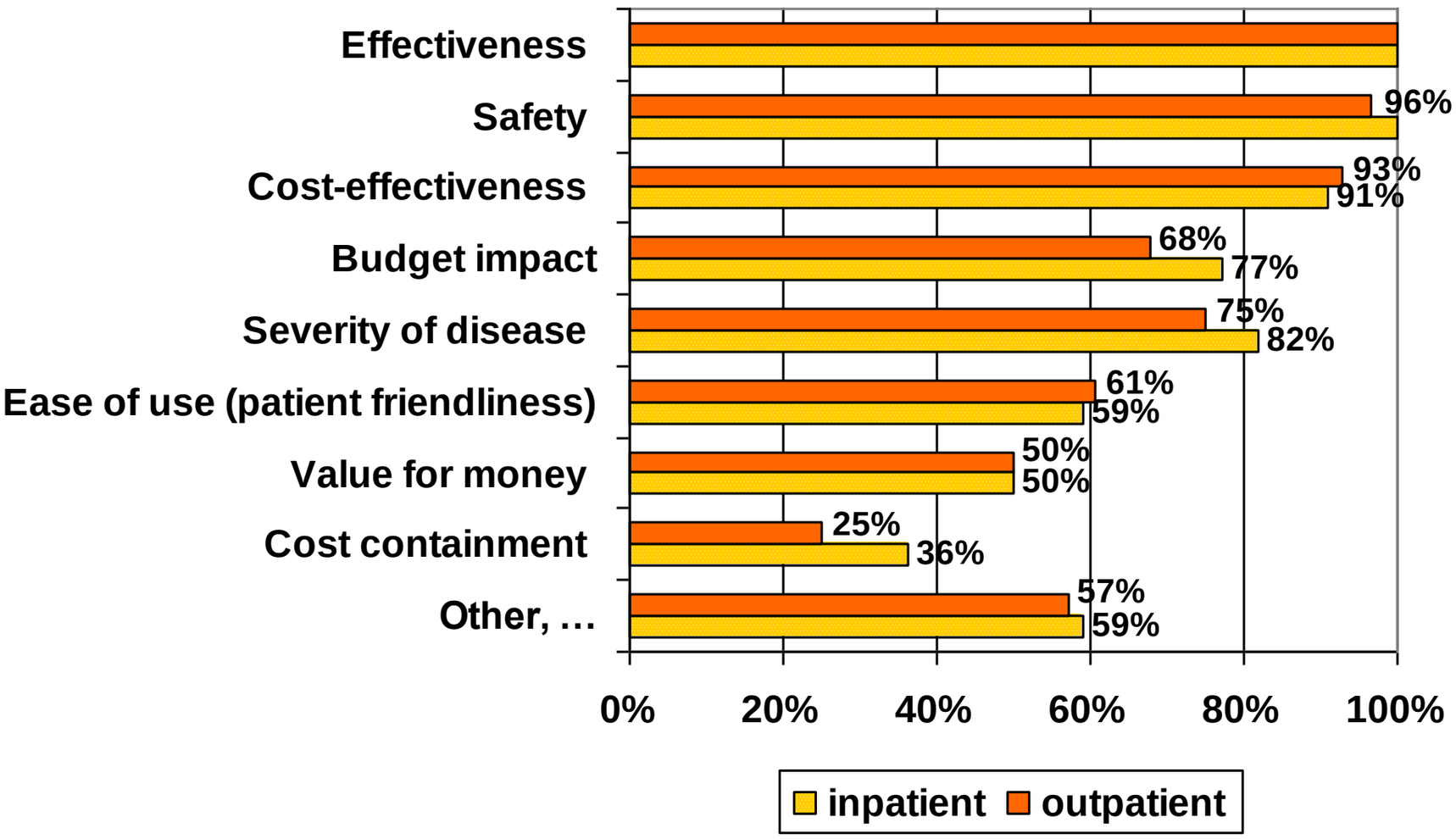
Background review

Criteria for reimbursement



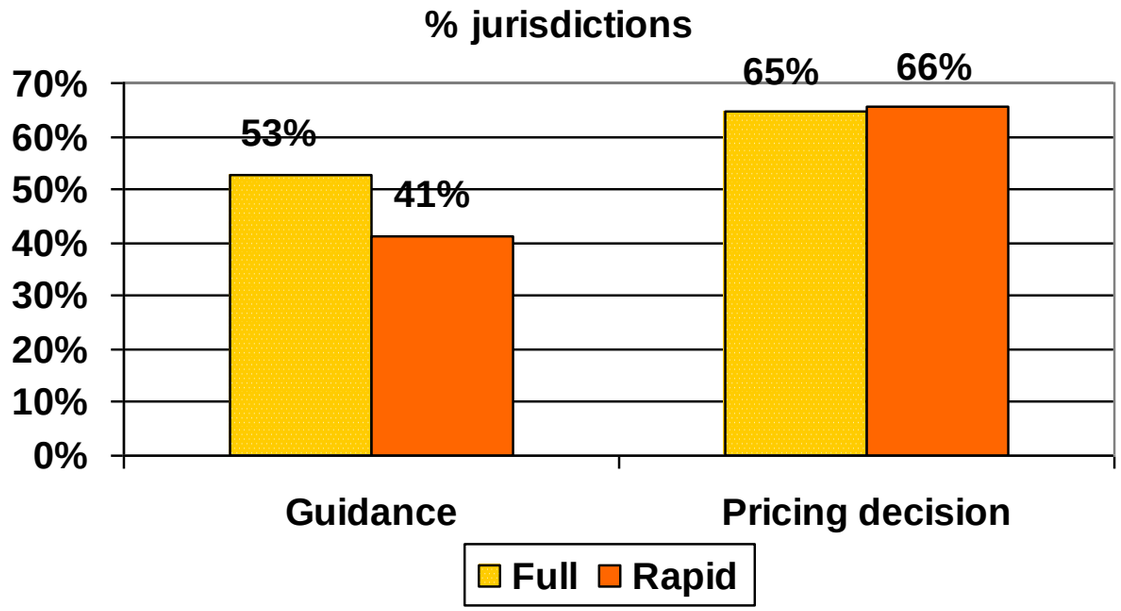
Number of jurisdictions included: 30

Background review

Rapid & Full assessment

- **% of jurisdictions that perform rapid/full assessment for <u>reimbursement</u> purposes:**
  - **rapid: 97%, of which 93% subject to timelines**
  - **full: 57%, of which 12 % subject to timelines**

- **<u>Other</u> purpose of assessment**



Number of jurisdictions included: 30

Background review

Some conclusions

- **Most of countries carry out some form of REA to support national reimbursement decisions of pharmaceuticals**

- **The scope and the methodology used vary across countries to some extent >> however not that much (TERMINOLOGY!)**

- **The differences between counties, as well as the reasons behind them, need to be considered in the development of a common European methodology for REA.**

- **Final report is published on EUnetHTA website:**
  - **http://www.eunethta.eu/Public/Work_Packages/EUnetHTA-Joint-Action-2010-12/EUnetHTA-JA-Public-Consultations/REA-Background-Review-public-consultation/**

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Background review

Stakeholder advisory groups (SAG)

- Input from experts from stakeholders (industry, patients, payers, healthcare providers)

- Before public consultation

- First involvement in background review
  - 77 comments provided by Europa Bio/ EDMA/ EFPIA/ EPF/ ESIP/ AESGP/ Eucomed
  - Very content related
  - **positive experience!**

Development of (rapid) REA model (including pilot)

HTA Core Model	WP5	
	Full Model	Rapid Model
Health problem and current use of technology	Health problem and current use of technology	Health problem and current use of technology
Description and technical characteristics of the technology	Description and technical characteristics of the technology	Description and technical characteristics of the technology
Safety	Safety	Safety
Effectiveness	Effectiveness	Effectiveness
Cost and economic considerations	~~Cost and economic considerations~~	~~Cost and economic considerations~~
Ethical analysis	Ethical analysis	~~Ethical analysis~~
Organisational analysis	Organisational analysis	~~Organisational analysis~~
Social aspects	Social aspects	~~Social aspects~~
Legal aspects	Legal aspects	~~Legal aspects~~
	•Mutliple comparators •Years after market authorisation •Indication based	•Limited number of comparators •Soon after market authorisation

WP5 pilot of a rapid assessment:

Objective of pilot

Test the usability of the draft rapid model for relative effectiveness assessment of pharmaceuticals and the draft guidelines

- **Primary outcome:**
  - the doers' perceptions about the model and guidelines (is the model structure helpful, is the guidance helpful in the assessment?)
- **Secondary outcomes:**
  - the duration of the assessment
  - the workload (in terms of working hours)
  - feasibility of international cooperation
  - the users' perceptions about the *format* of the assessment report, adaptability of information into national purposes, and its readability.

Disclaimer: the results of the assessment should be handled with care and are not suitable to draw conclusions for decision making.

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WP5 pilot of a rapid assessment:

Topic selection & Basic documentation

Topic selection:

- **List was produced of all pharmaceuticals that received market authorization between June 2010 and February 2011**
- **Selection made based on exclusion criteria**
  - List of eight pharmaceuticals was sent to all WP5 members >> asked to state their preference
  - Shortlist of 4 most preferred pharmaceuticals send to SAG and EMA >> no objections
- **Manufacturers approached for willingness to provide submission file (2 out of 4 were willing)**
  - **Pazopanib for the first-line treatment of metastatic renal cell cancer.**

Basic documentation:

- **Manufacturer submission file**
- **Rapid REA model**
- **Methodological guidelines developed in WP5**

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WP5 pilot of a rapid assessment:

Organisation

- **Project manager & coordinator(s)**
- **Coordination team:**
  - domain leads, project coordinator & manager
- **Per domain (8 domains)**
  - Domain lead (8)
  - Authors (26 including domain leads)
  - Reviewers (18)
- **Synthesis team: compiles relative effectiveness section**

- **Participants: 29 HTA organisations (all WP5 members) from 14 European countries**

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WP5 pilot of a rapid assessment:

Project phases & timelines

Producing the draft report:

- Protocol phase (May-June 2011)
- Assessment Phase (June-October 2011)

>> Despite the large number of participants and the short timelines almost all domains met their deadlines indicating willingness to participate!

Consultations

- Pilot doers/ WP5 and manufacturer (nov/dec 2011)
- Stakeholder advisory group (feb/ma 2012)
- Public consultation (apr/may 2012)

>> The consultations are essential to safeguard quality and stakeholder involvement however they have a huge impact on the timelines of producing a final report!

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WP5 pilot of a rapid assessment:

Structure of (draft) report

- **Summary**
  - Scope
  - Relative effectiveness section
  - Summary of domain reports
- **Introduction**
  - objective/topic selection/oganisation
- **Methods**
  - Basic search/scoping/ selection research questions……
- **Domain reports**
  - 8 domains
  - Per domain: domain specific methods/summary of results/discussion/assessment elements
- **Discussion**
    - **On primary outcomes of pilot > based on input from consultations**

WP5 pilot of a rapid assessment:

Lessons learned so far?!

- **Participation of 29 organisations requires intense coordination on several levels**
  - In domains
  - Between domains
  - Overall scope
- **3,5 months timelines for scope/assessment phase if possible but very intense**
- **Relevance of all research questions (assessment elements) for a rapid assessment?!**
  - Number of domains (8) and/or number of assessment elements for rapid assessment can/should be reduced?!

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Collaboration with EMA

- **Adaptation of EPAR (2011)**
- **Evaluation of adapted EPAR (2011-2012)**
- **Consultation of guidelines developed for REA of pharmaceuticals (2012-….)**
  - Consistency between EUnetHTA and EMA guidelines?
- **Collection of post-marketing data (ENCEPP, EVIDENT (WP7)) (2012-….)**

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