Why is Health Technology Assessment Important?

Joint BBS and EFSPI HTA Seminar

Allschwil, June 4, 2013

Xcenda
AmerisourceBergen Consulting Services

Acknowledgements

Tommy Bramley
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Eric Wery

In Conclusion… (from November 2012)

- Foster a strong collaboration between Clinical R&D, Drug Safety, Health Economics and Marketing with strong biostatistics (quantitative) support in all areas
- Define clear roles and responsibilities to make the most effective use of expertise, skills and resources
- Contribute more case studies on how methodologies are best applied and influence decision making
- Enable effective communication of value evidence generation activities across the whole product life-cycle
- Provide for early engagement and cross-functional alignment on regulatory and market access hurdles
- Be flexible and adaptable to meet a complex and evolving global market environment and still meet needs of patients with best available cost-effective care

Preferred Definitions Differentiate Among EBM, CER, and HTA

EVIDENCE-BASED MEDICINE (EBM)

- EBM is an evidence synthesis and decision process used to assist patients' and/or physicians' decisions.
- It considers evidence regarding the effectiveness of interventions and patients' values and is mainly concerned with individual patients' decisions, but is also useful for developing clinical guidelines as they pertain to individual patients.

COMPARATIVE EFFECTIVENESS RESEARCH (CER)

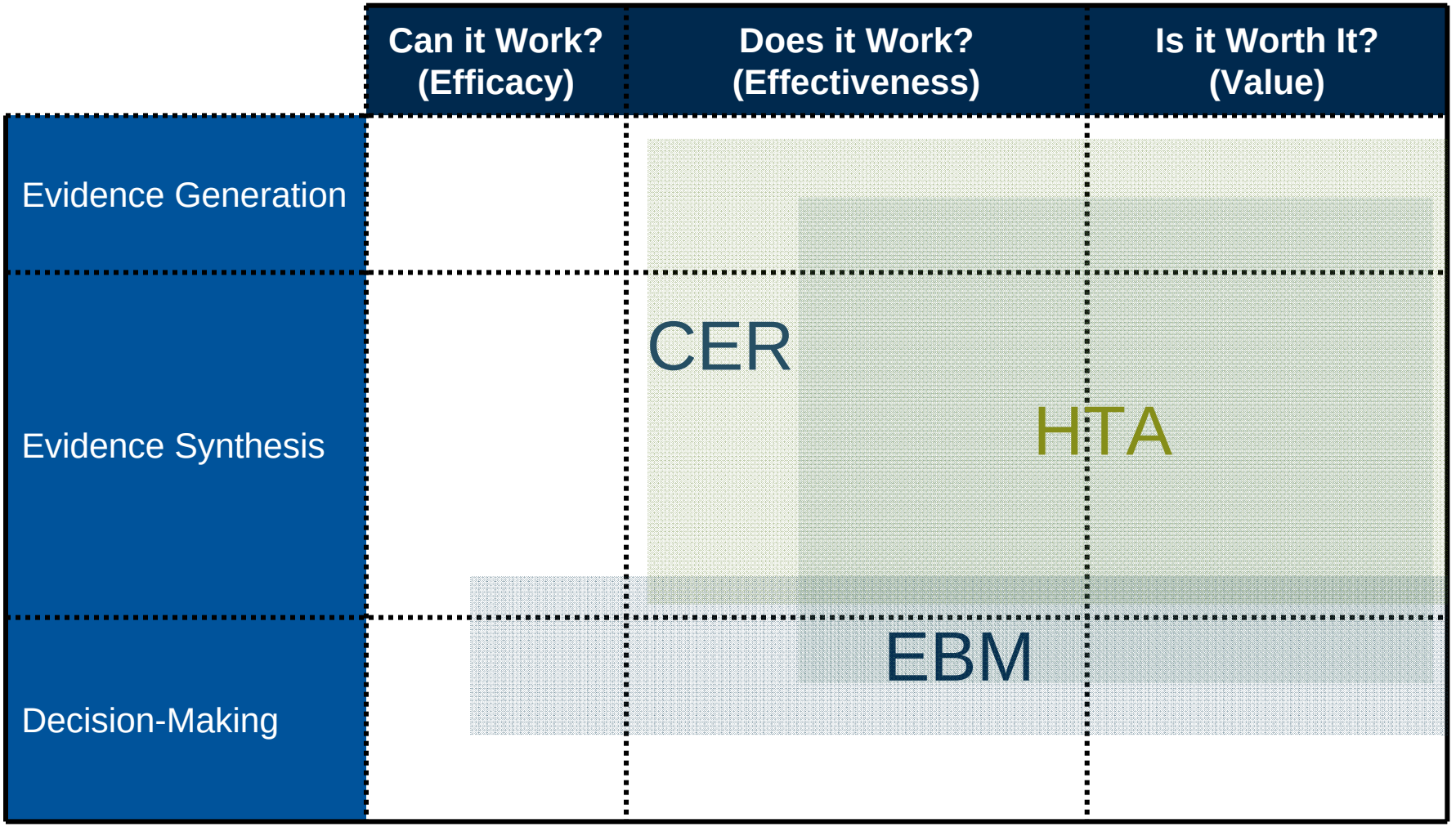
- CER includes both evidence generation and evidence synthesis.
- It is concerned with the comparative assessment of interventions in routine practice settings.
- The outputs of CER activities are useful for clinical guideline development, evidence-based medicine, and the broader social and economic assessment of health technologies (i.e., HTA).

HEALTH TECHNOLOGY ASSESSMENT (HTA)

- HTA is method of evidence synthesis that considers evidence regarding clinical effectiveness, safety, cost-effectiveness, and, when broadly applied, includes social, ethical, and legal aspects of the use of health technologies.
- A major use of HTAs is in informing reimbursement and coverage decisions, in which case HTAs should include benefit-harm assessment and economic evaluation.

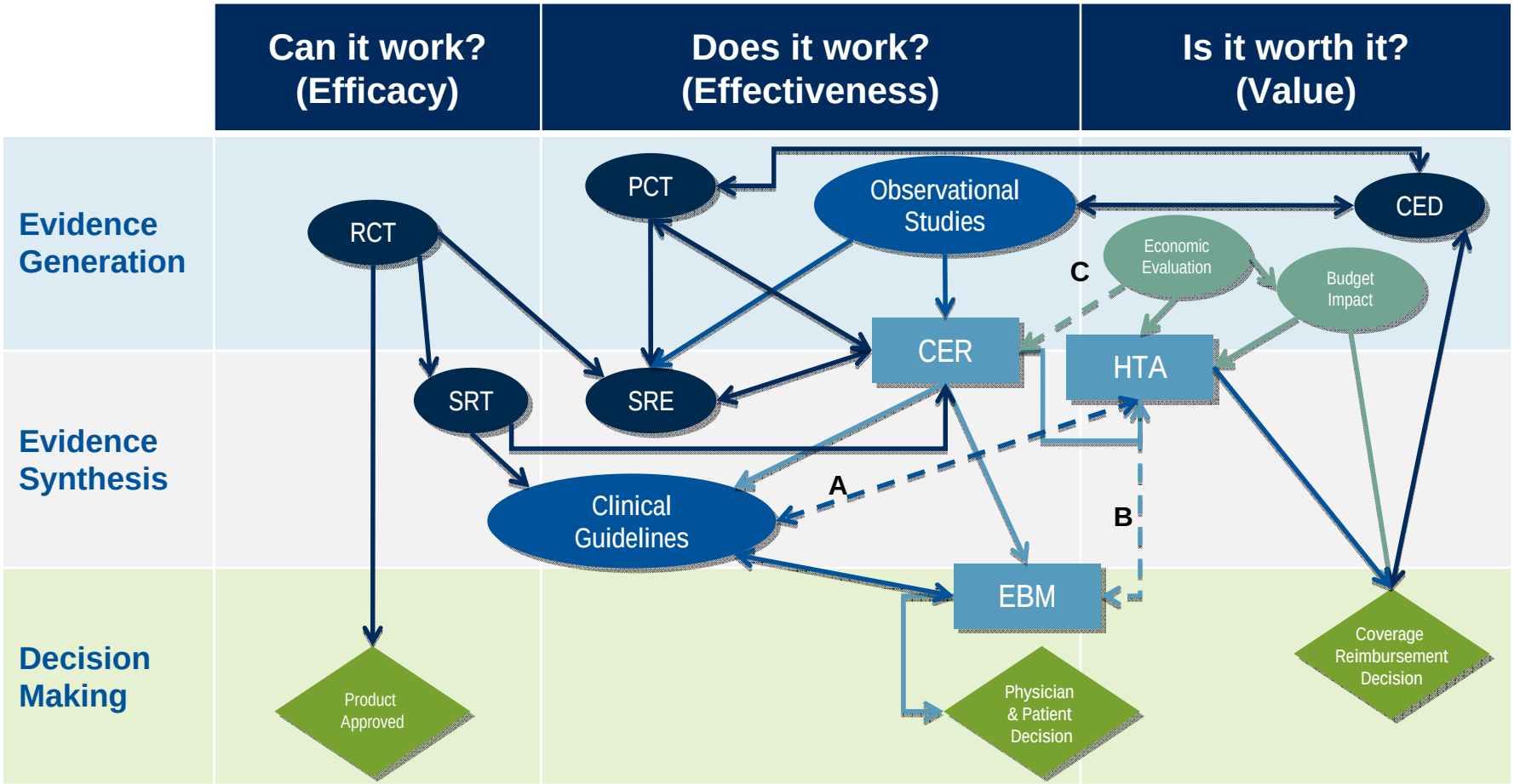
1. Luce BR, Drummond M, Jönsson B, et al. *Milbank Quarterly*. 2010;88(2):256-276

Confusion Exists Concerning Appropriate Definitions of CER, HTA, and EBM



1. Luce BR, Drummond M, Jönsson B, et al. *Milbank Quarterly*. 2010;88(2):256-276.

Redefined Relationships of Evidence Processes



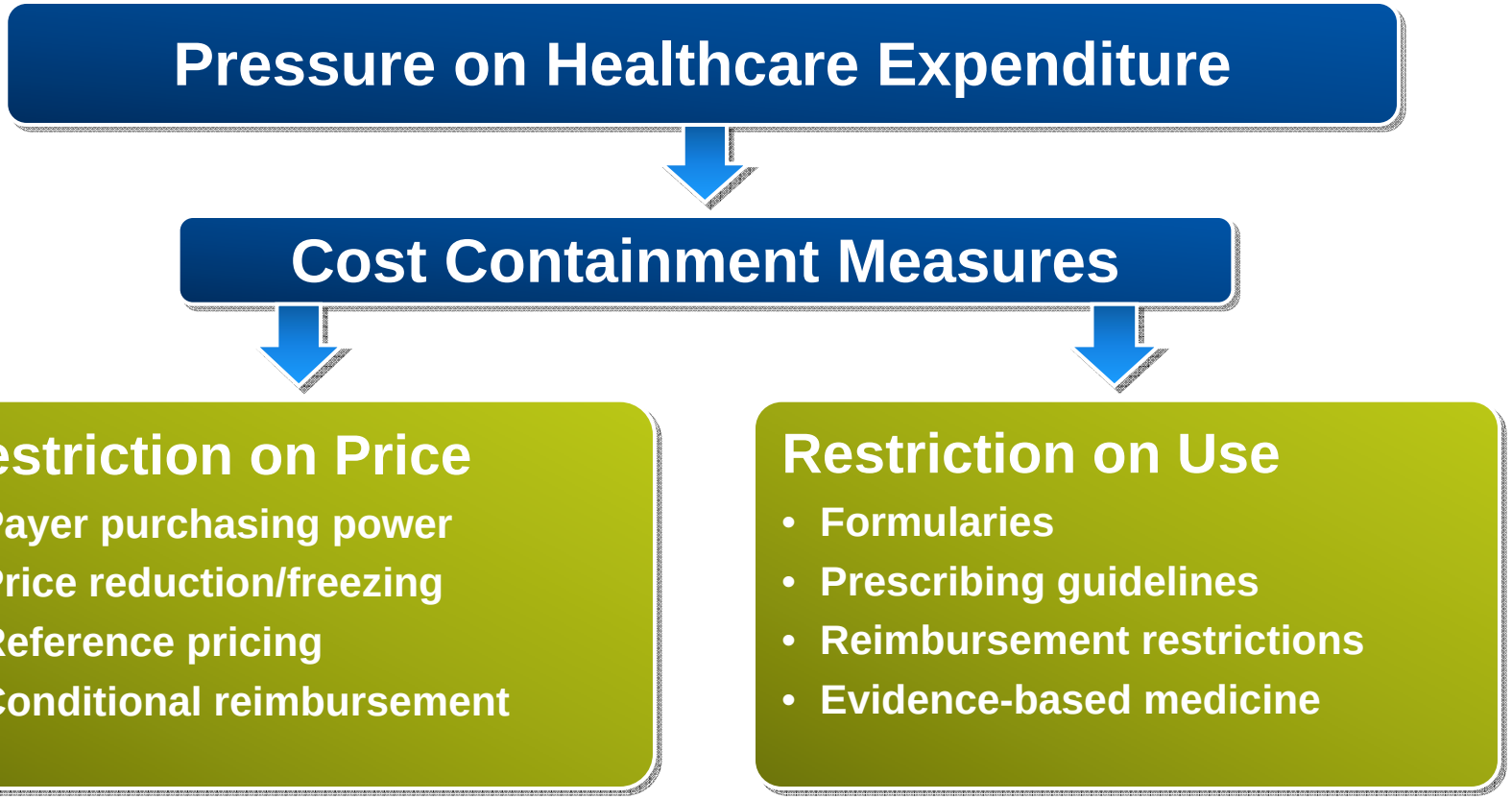
RCT – randomized controlled trial; PCT – pragmatic clinical trial; SRT – systematic review of trials; SRE – systematic review of evidence; CER – comparative effectiveness research; HTA – health technology assessment; EBM – evidence-based medicine; CED – coverage with evidence development.

Solid lines indicate clear relationships, and dotted lines indicated disputed relationships. Diamonds represent decision processes, and circles and ovals represent all other evidence activities, except for the rectangles, which are reserved for EMB, HTA, and CER.

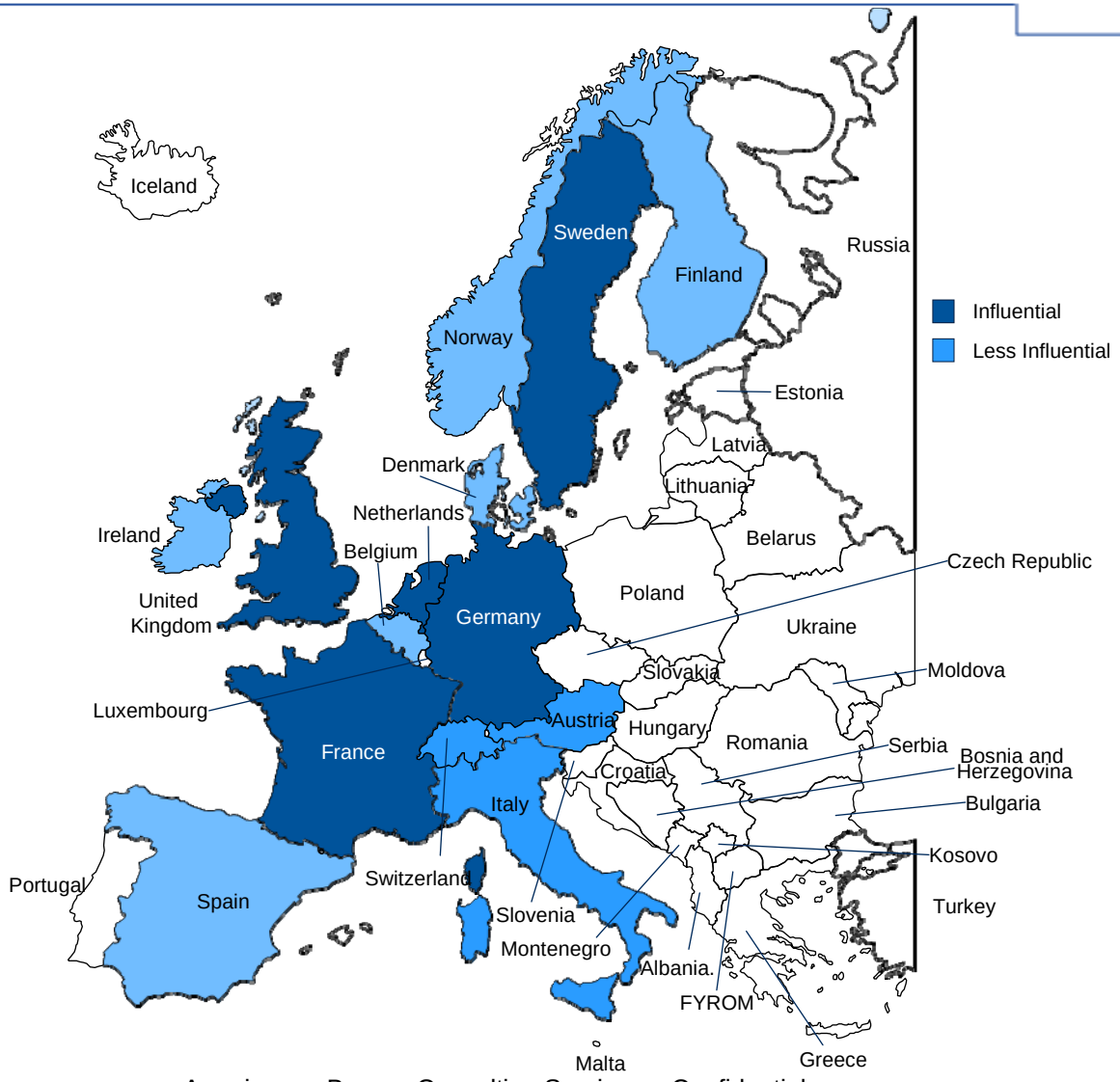
Pricing, Reimbursement, Access

- Pricing is the process of securing a price, usually listed, with a payer
- Reimbursement is securing a payer's funding
- Access is securing product availability in the market with 'use' cost containment measures
- A higher price often implies greater 'use' cost containment measures
- Operationally, reimbursement can refer to specific customer support to secure access for a patient or local institution
  - Training, assistance with submitting paperwork
  - Coding issues
- Pricing, reimbursement, and access depend on evidence of value
  - Evidence of value may be different depending on country, but also at regional or local levels within a specific country

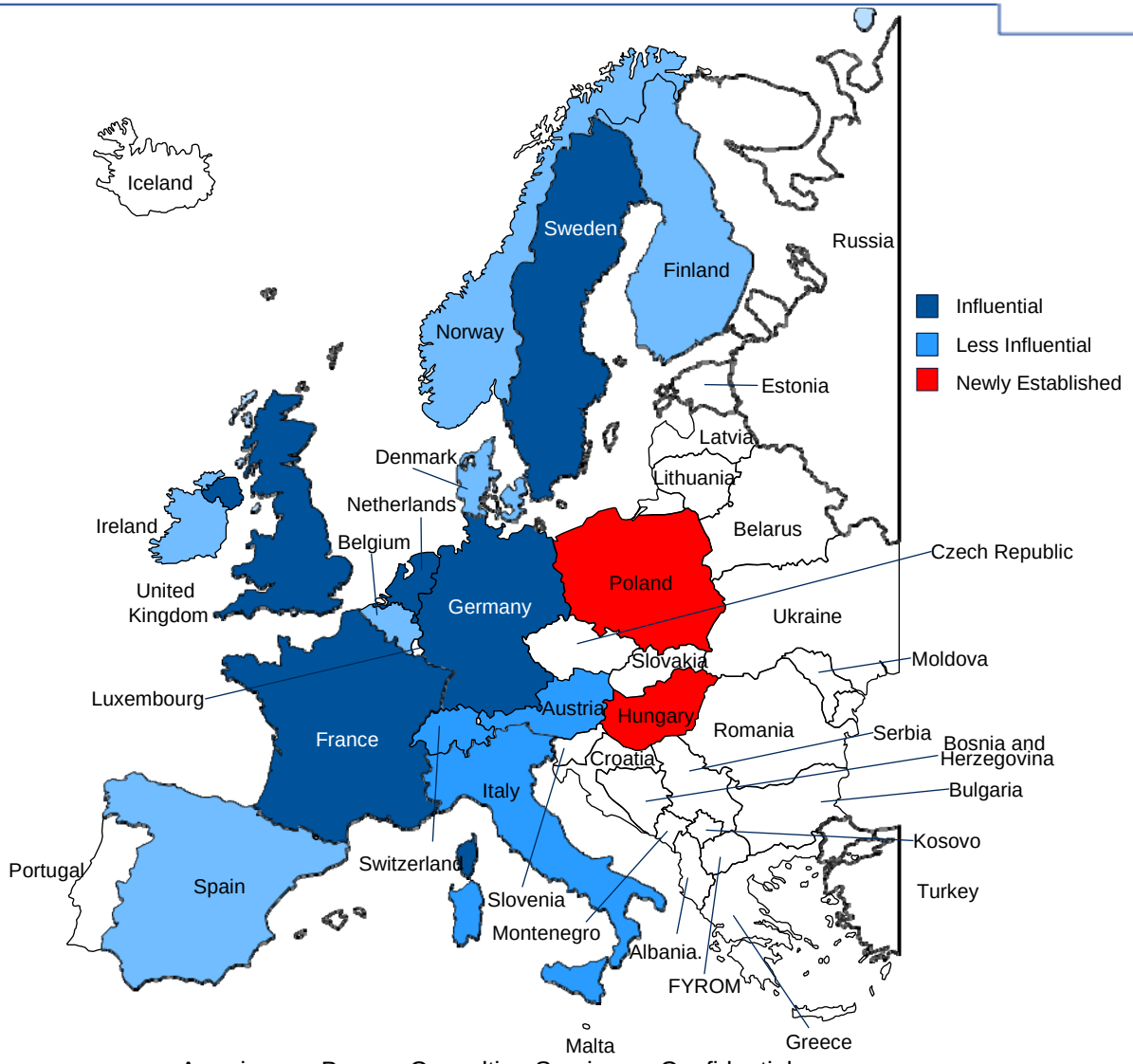
Increasing Costs Are Met With Cost Containment Measures



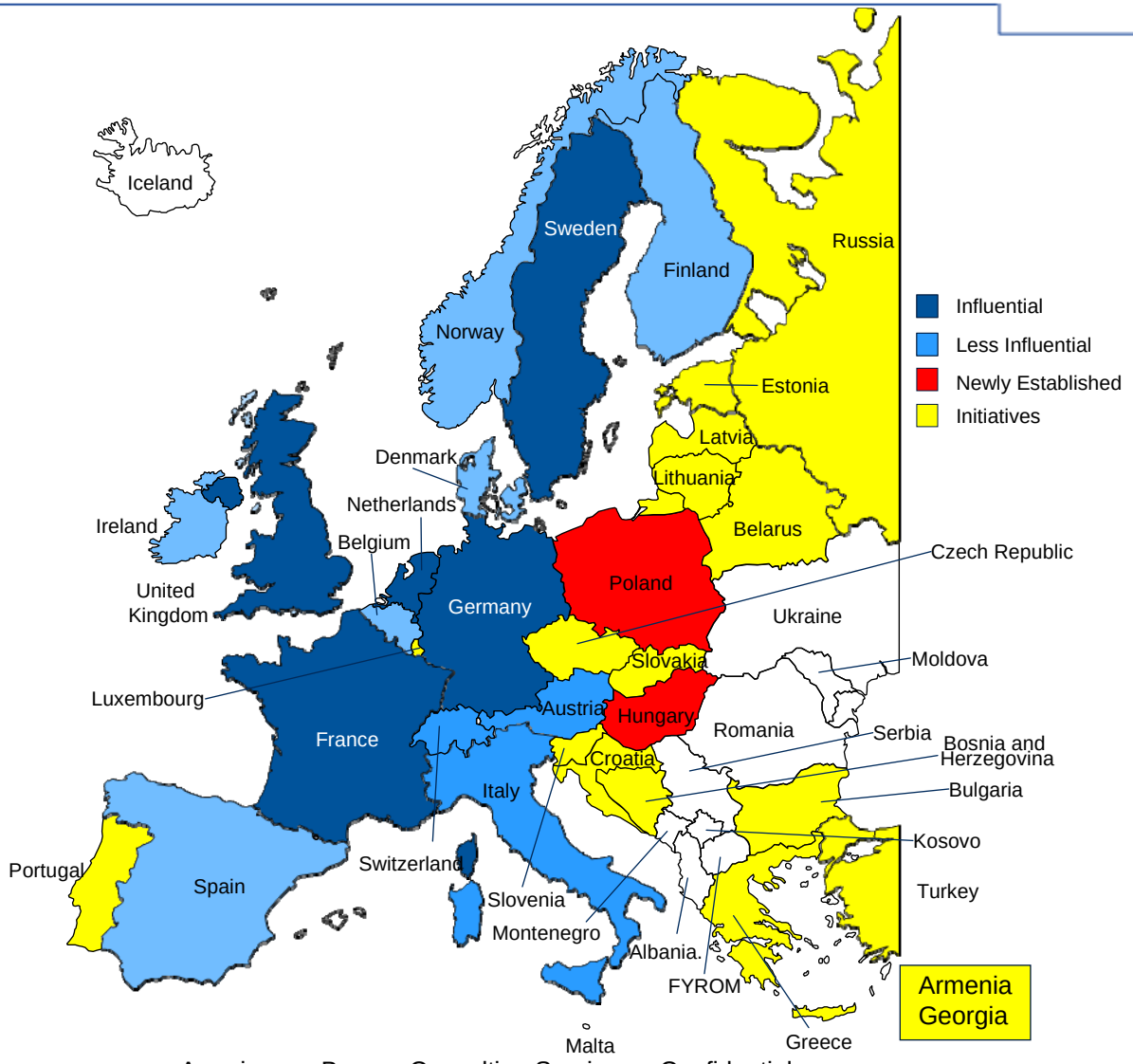
Formal HTA Bodies in Europe



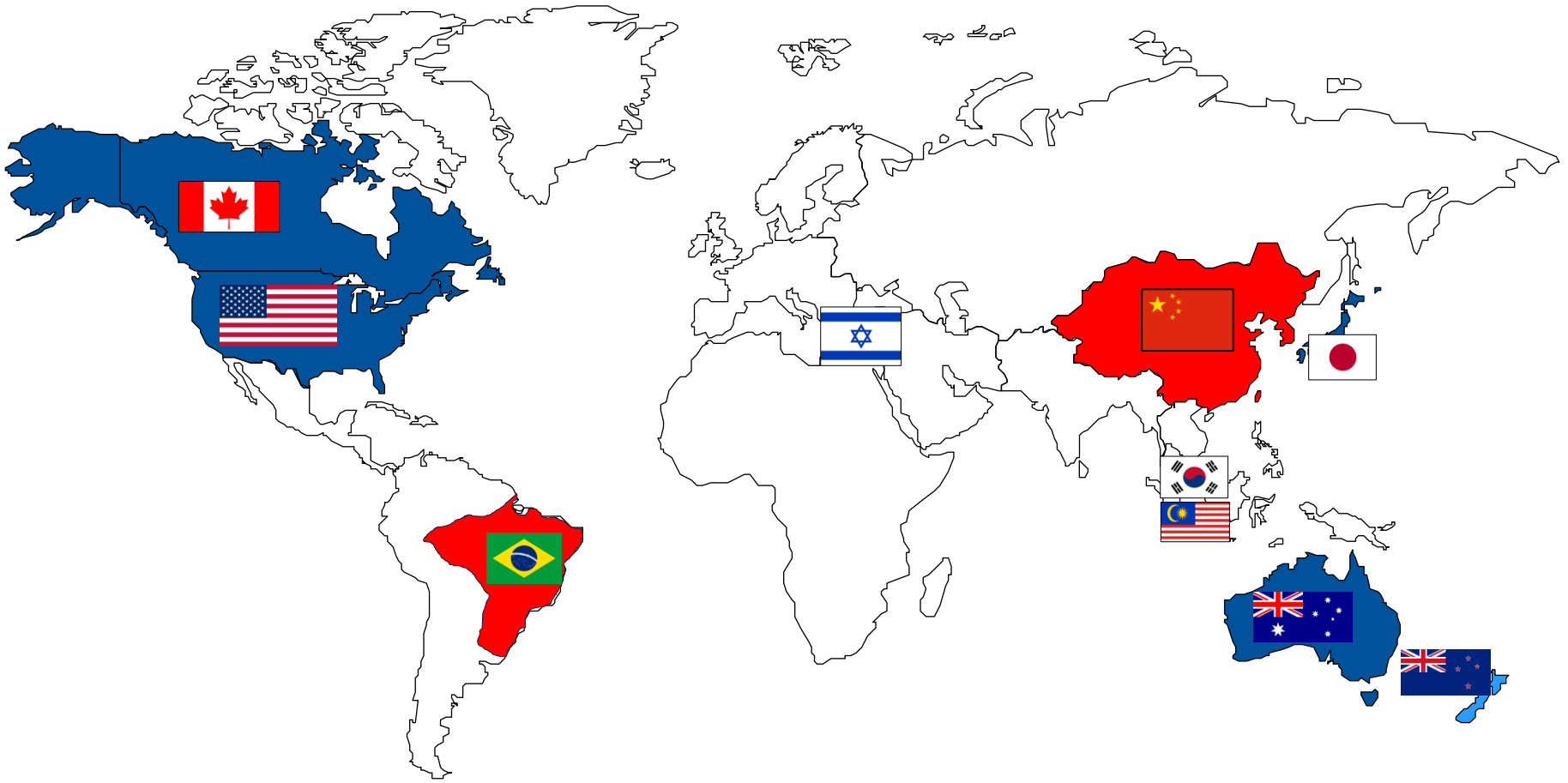
More Recently Established Bodies



Emerging Initiatives



Recent Major Developments

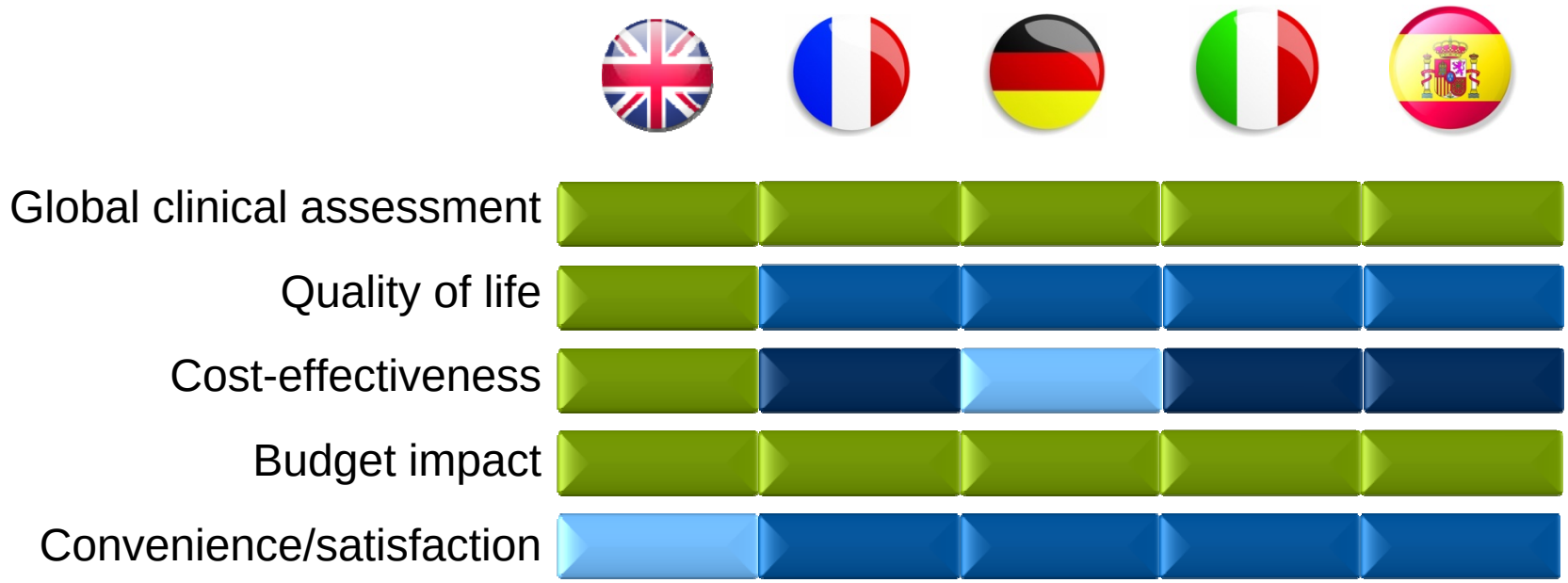


Key Learning

THE HTA WORLD IS CHANGING

- Even though a specific country does not perform formal HTAs today does not mean it will not do so in a few years
- Even though a specific HTA body does not ask for a specific type of evidence today does not mean it will not do so in a few years
- Take into consideration the fact that you will need to prepare for HTA review in an increasing number of geographies
- **REMAIN AWARE OF CHANGES AND PLAN FOR THEM**

HTA Requirements – General Topics



Key Learning

GENERATE THE RIGHT TYPE OF EVIDENCE

- Evidence requirements differ between HTA agencies
- Evidence has to be gathered at all stages of the product development and after launch
- Evidence must be in line with your value message
- **GENERATE THE EVIDENCE THAT WILL ALLOW YOU TO SUBSTANTIATE THE PRODUCT VALUE PROPOSITION**

Reform of the Major HTA Systems

The major European HTA bodies are currently all going through major reforms

NICE

Value-based Pricing vs Free Pricing

- Pricing will be increasingly based on evidence – end of free pricing
- More restrictive application of cost-effectiveness by different thresholds and by indication
- More focus on severity of disease and impact of treatment
- Patient access schemes

IQWIG

Ongoing AMNOG Reform

- Free pricing for 1 year only and negotiations
- Major focus remains on clinical evidence from randomized trials
- Added clinical benefit demonstration will be critical

HAS

Reform of the Appraisal Process

- New appraisal will be a mix between NICE and IQWIG processes
- Focus on added clinical benefit
- Greater importance of economic evidence components

Some general trends:

- Increasing demand for evidence of added value
- More focus toward economic impact
- More pressure on price

Key Learning

MAJOR SYSTEMS ARE CHANGING

- The major HTA systems are going through significant reforms
- The demand for evidence is increasing and evidence generation needs to be carefully planned
- Price pressure is increasing, and obtaining a premium price is strongly linked with premium evidence
- **REMAIN AWARE OF THE CHANGES IN YOUR MAJOR MARKETS AND BE PREPARED TO GENERATE THE APPROPRIATE EVIDENCE TO SATISFY THEM**

Key Learning – Summary

THE HTA WORLD IS CHANGING

- Remain aware of changes happening in the global market and prepare for them

GENERATE THE RIGHT TYPE OF EVIDENCE

- Generate the evidence that will allow you to substantiate the product value proposition

MAJOR SYSTEMS ARE CHANGING

- Remain aware of the changes in your major markets and be prepared to generate the appropriate evidence to satisfy them

Thank You!

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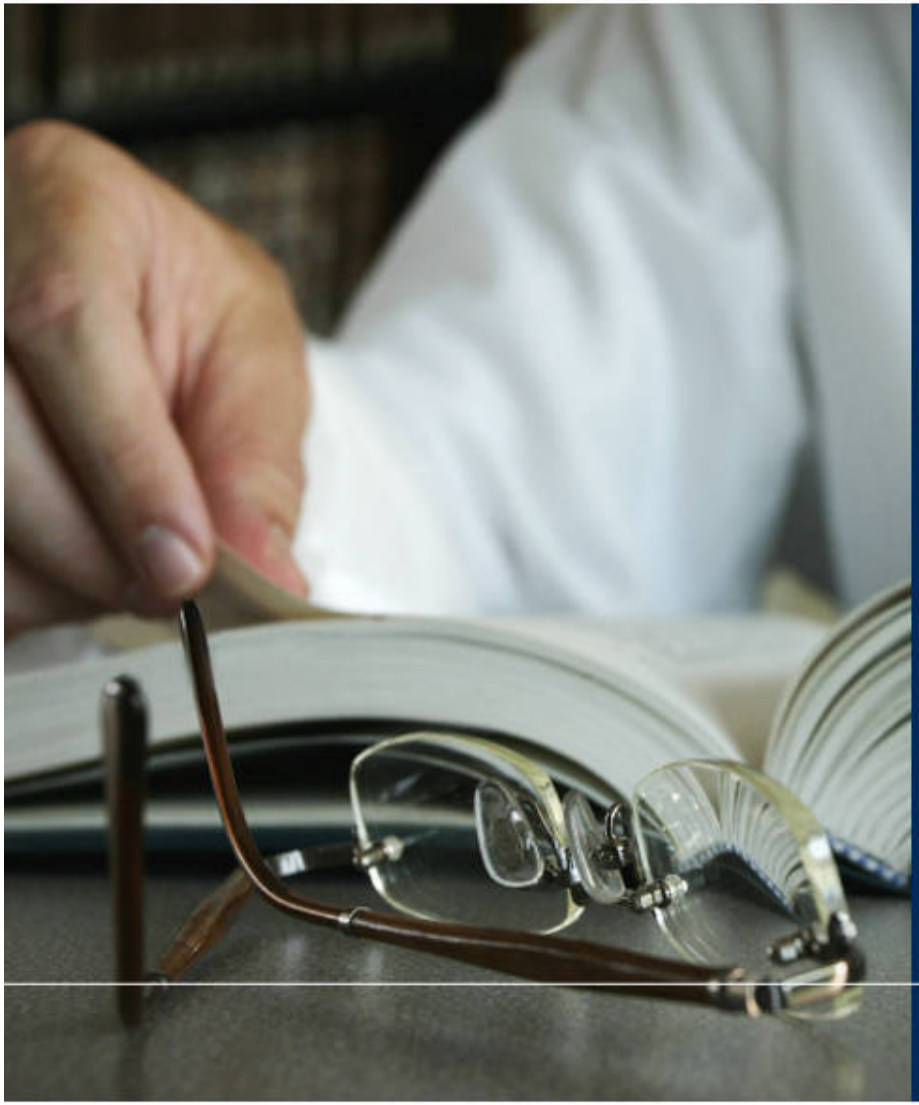


Comparative Effectiveness in the United States

Different Priorities and Evidentiary Requirements

Key US Payer Insights Provided by Xcenda's Managed Care Network

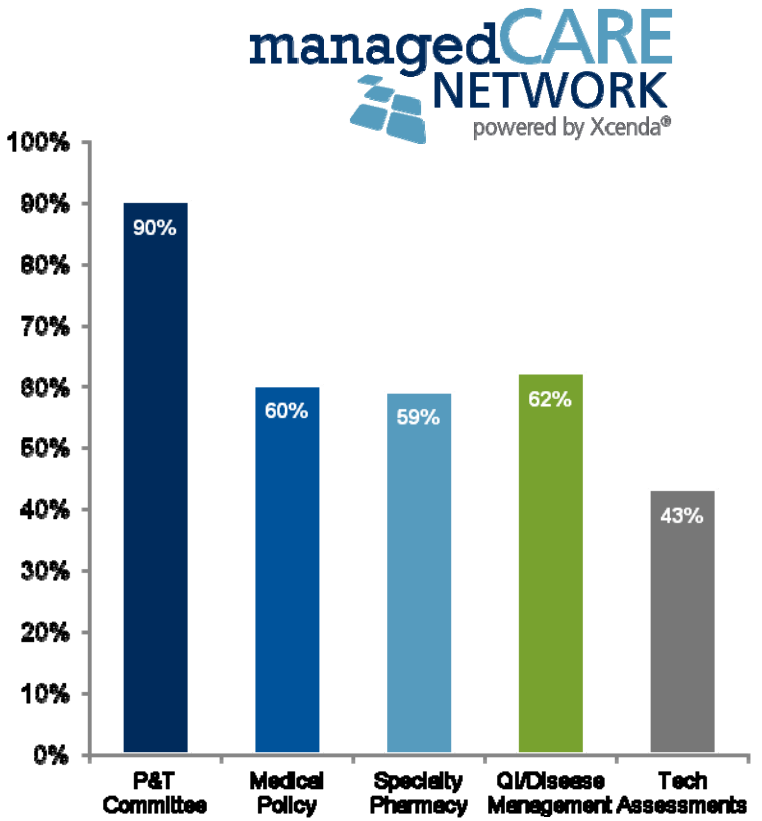
MCN Advisors' Primary Decision-maker Roles

Advisor experience

- Average of 25 years in practice
- Average of 9 years with current organization

Advisor titles

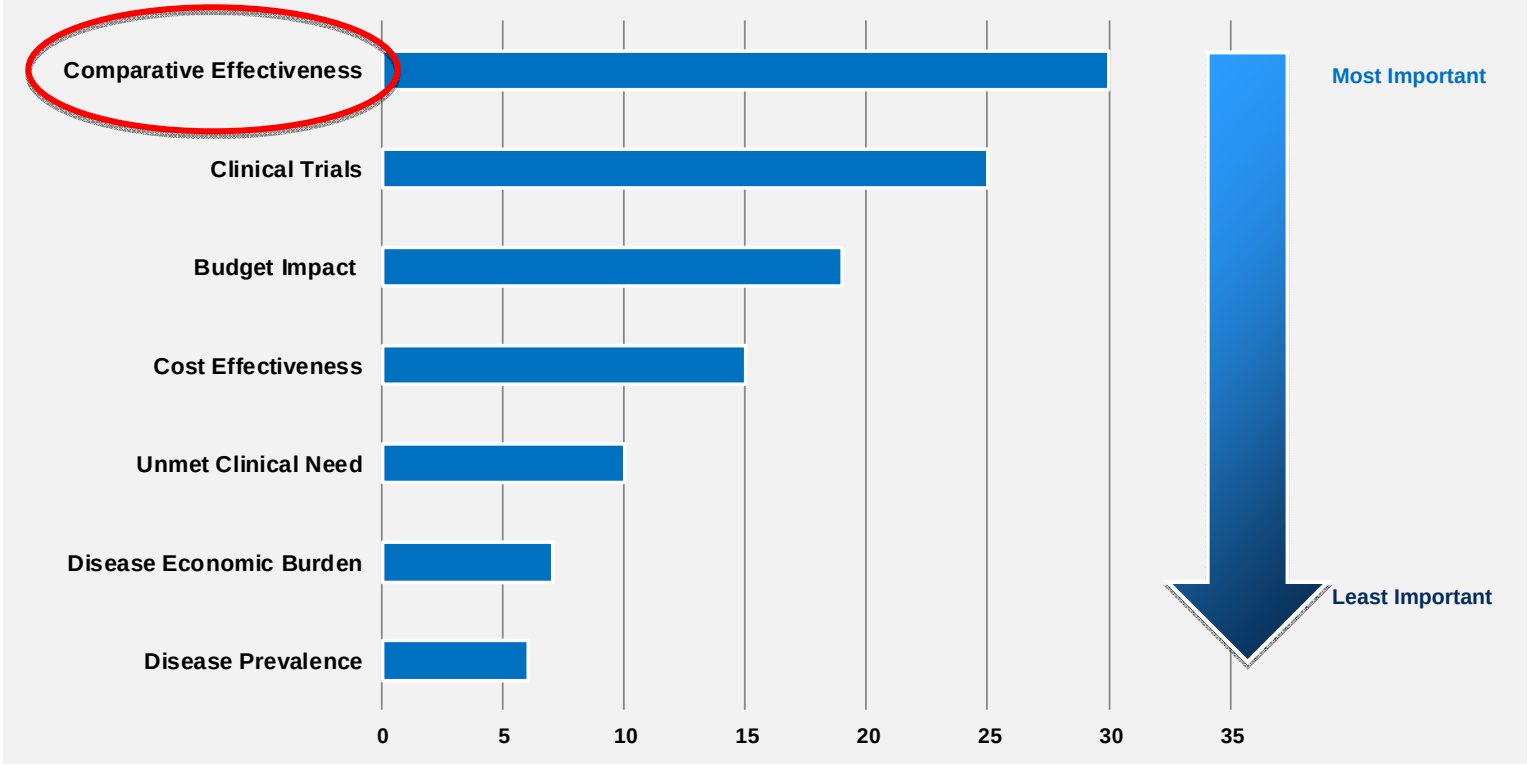
- Executive (CMO, CPO, VP)
- Pharmacy Director
- Medical Director
- Clinical Manager / Director
- QI Director
- Consultant



N=110 advisors (130 million MCO/IHDN lives and 60 million PBM/SPP lives).

Payer Insight
Comparative Effectiveness Data

Importance of Various Types of Information on Formulary Evaluation

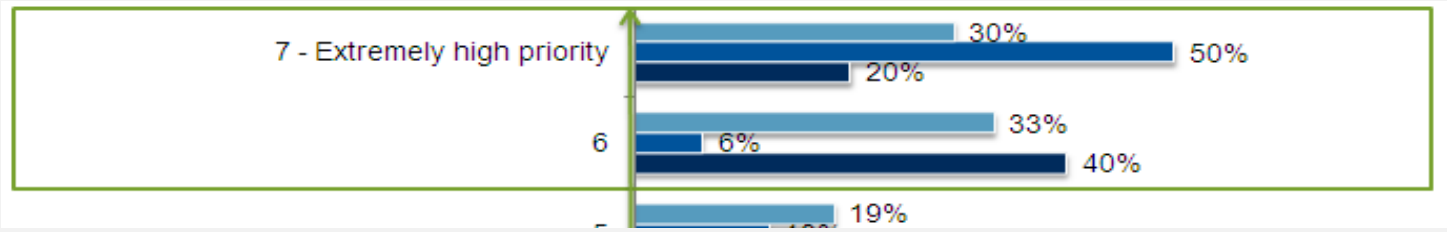


Number of ratings of "very important"; max=46.
Source: Xcenda Managed Care Network Survey (n=46).

Payer Insight
Safety

Xcenda PayerPulse® Survey, 2012

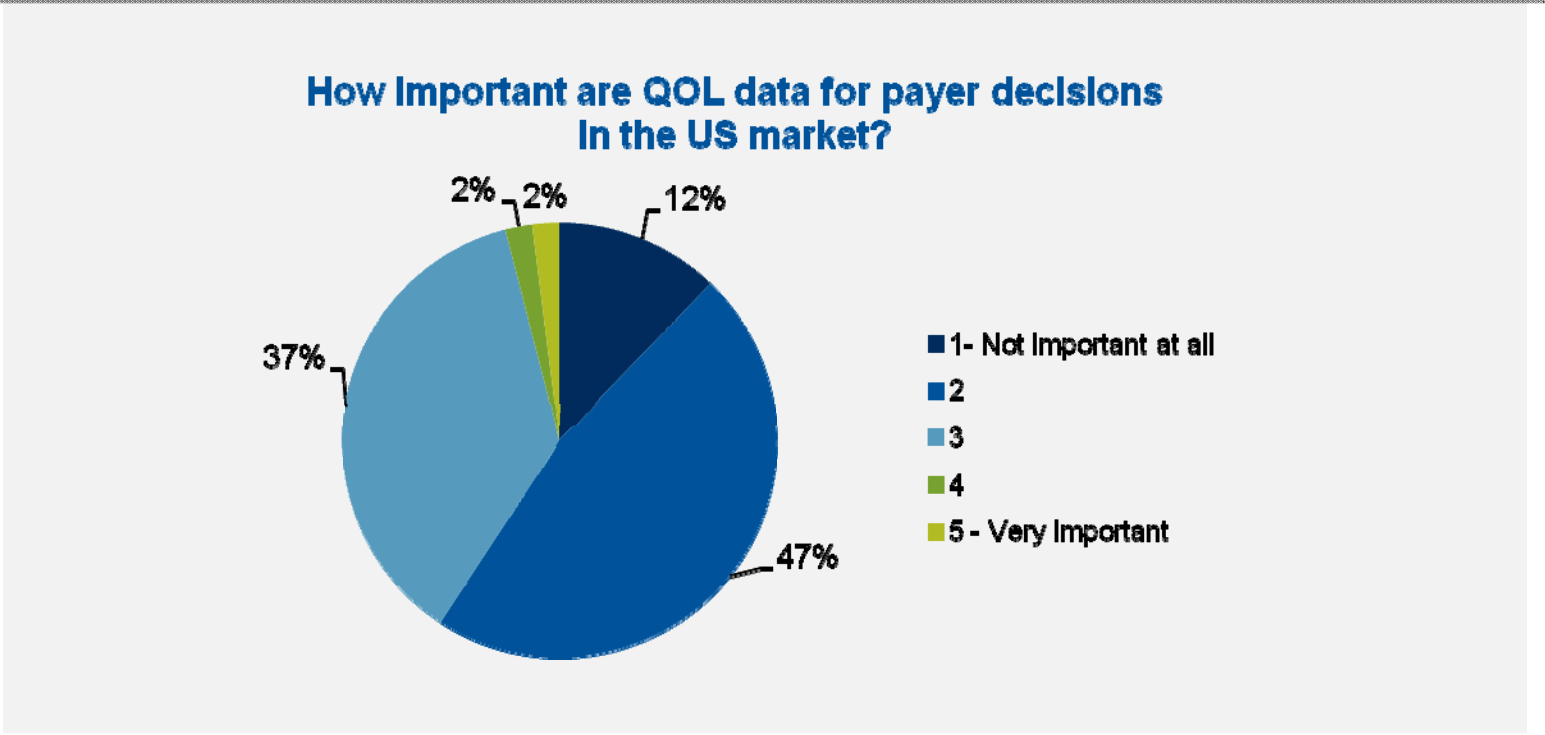
"Nearly two-thirds (63%) of pharmacy directors and over half (56%) of medical directors rated *cost of hospitalizations* as a moderately to extremely high priority for their plan"



N=48 respondents; 117 million covered lives.

Payer Insight
Quality of Life (QOL)

Xcenda PayerPulse Survey, 2010

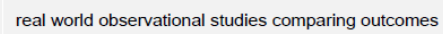


N=49 respondents; 114 million covered lives.

Payer Insight
Beneficial Study Designs

Xcenda PayerPulse Survey, 2010

6. Clinical trials are currently designed to meet FDA approval requirements for efficacy and safety. Beyond these regulatory requirements for safety and efficacy, which of the following types of study designs would be beneficial to your plan's coverage decisions? (Choose all that apply)	Response Percent	Response Total
a. Observational studies using secondary data (e.g. retrospective claims and administrative data analyses)	14.3%	7
b. Comparative studies using non-randomized designs (e.g. case control, cohort studies)	10.2%	5
c. Head to head evaluations against comparator drug (prospective, non-randomized)	83.7%	41
d. Randomized control trial against comparator (effectiveness trial)	77.6%	38
e. Other	2%	1



N=49 respondents; 114 million covered lives.

Payer Insight

Impact of HEOR Evidence on Formulary Decisions

Xcenda PayerPulse Survey, 2012

	1 - No impact on formulary decisions	2	3	4 - Neutral	5	6	7 - Greatest impact on formulary decisions	6 & 7 combined
Prospective real-world studies	0%	2%	5%	7%	32%	42%	13%	55%
Adaptive trial designs (prospective, concurrent, and retrospective)	0%	2%	3%	19%	44%	25%	7%	32%
Retrospective real-world studies of electronic medical record data	2%	5%	7%	19%	37%	25%	5%	30%
Retrospective real-world studies of administrative claims data	5%	3%	12%	15%	36%	29%	0%	29%
Meta-analyses	2%	5%	17%	14%	48%	14%	2%	16%
Retrospective real-world studies of survey data	5%	10%	22%	27%	27%	9%	0%	9%
Combining individual patient data from clinical trials	2%	2%	15%	32%	41%	5%	3%	8%

N=59 respondents; 135 million covered lives.

Payer Insight
Desirable Data

How do: 1) analytic validity, 2) clinical validity, and 3) clinical utility stack up with US payers?

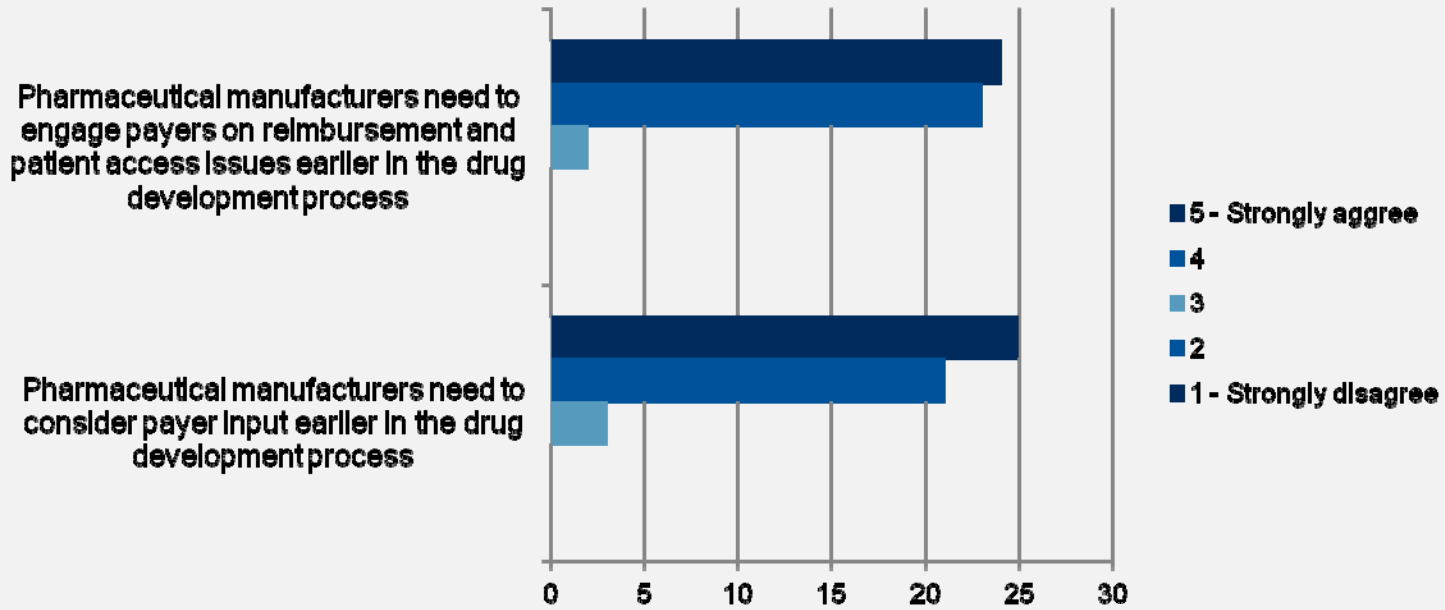
	1 & 2 combined (1 = no impact at all)	3, 4, 5 combined (4 = neutral)	6 & 7 combined (7 = extremely impactful)
Lack of clinical utility; whether providers will use the test results to drive treatment decisions and/or how patients will be managed	3%	40%	56%
Lack of evidence of test accuracy	5%	39%	56%
Insufficient clinical data; not enough known on actual value and associated risk of treating	3%	49%	47%
Lack of economic data net savings or cost increase	2%	57%	42%
Lack of prevalence data (number needed to test, number needed to treat)	2%	61%	37%
Poor estimation of the cost of misclassification (ie, not treating appropriate patients or overtreating inappropriate patients)	2%	67%	32%
Others not listed: 1) label; 2) comparative effectiveness research; 3) overall survival; 4) patient/provider acceptance	18%	64%	18%

N=59 respondents; 135 million covered lives.
Source: Xcenda PayerPulse Survey 2012.

Payer Insight

Payer Decision Makers Want to Be Involved in Research Design

Xcenda PayerPulse Survey, 2010



N=49 respondents; 114 million covered lives.
Source: Xcenda PayerPulse Survey 2012.

The Future Is Now…
Linking Drug Payment to Health Outcomes

Program/Partners	Agreement Type	Outcome
Lucentis/ National Health Service	Dose cap at 14 injections, after which drug company pays for product	Clear criteria for reimbursement
Actonel/ Health Alliance	Drug company gives rebate to health plan based on fractures incurred while patients are on the drug	Need for further data collection and coordination with health plan
Januvia and Janumet/ Cigna	Drug company discount is increased if HbA1c values improve in 1 year for patients on any oral diabetes therapy	Outcomes are attributed to multiple glucose-lowering medications and not solely to Januvia/Janumet
Velcade/ National Health Service	Drug company reimburses insurer for the first 4 cycles of treatment with no patient response	Administrative burdens when tracking patients who respond
Beta-Interferons/ National Health Service	Initial discount plus price adjustments if results are 20% more or less than initially projected over 10 years	Measuring effects are difficult because of varying course of the disease; inter-rater reliability is also poor

Health Affairs, 2011.

Thank You!

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