



Optimizing the Utility of Evidence from Multiple Sources in Oncology Outcomes Research

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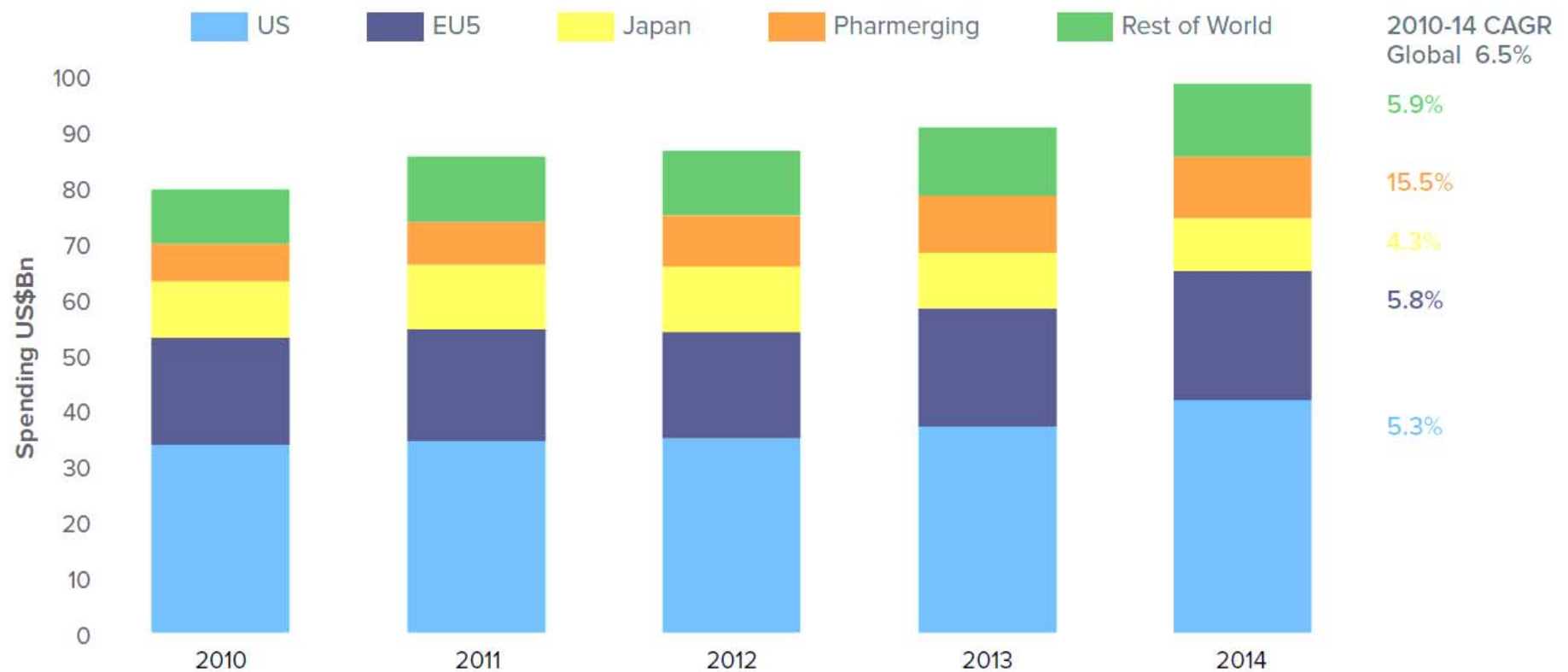
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Global oncology spend has grown to \$100 billion in 2014

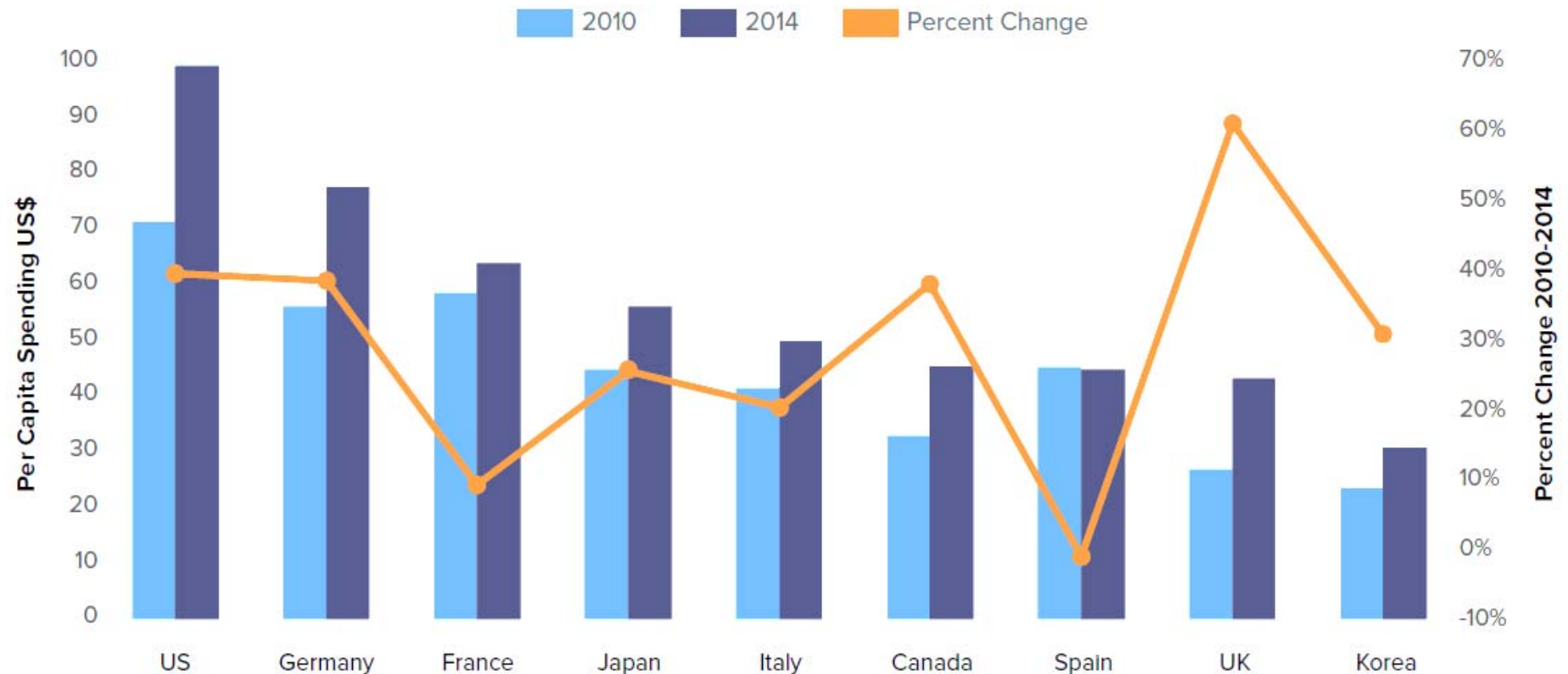


Source: IMS Health MIDAS, Dec 2014

Reference: A

- US continues to dominate the market with 42% of total spend
- CAGR for Pharmerging countries are higher than rest of the world

Per capita oncology drug spending increased in most developed countries

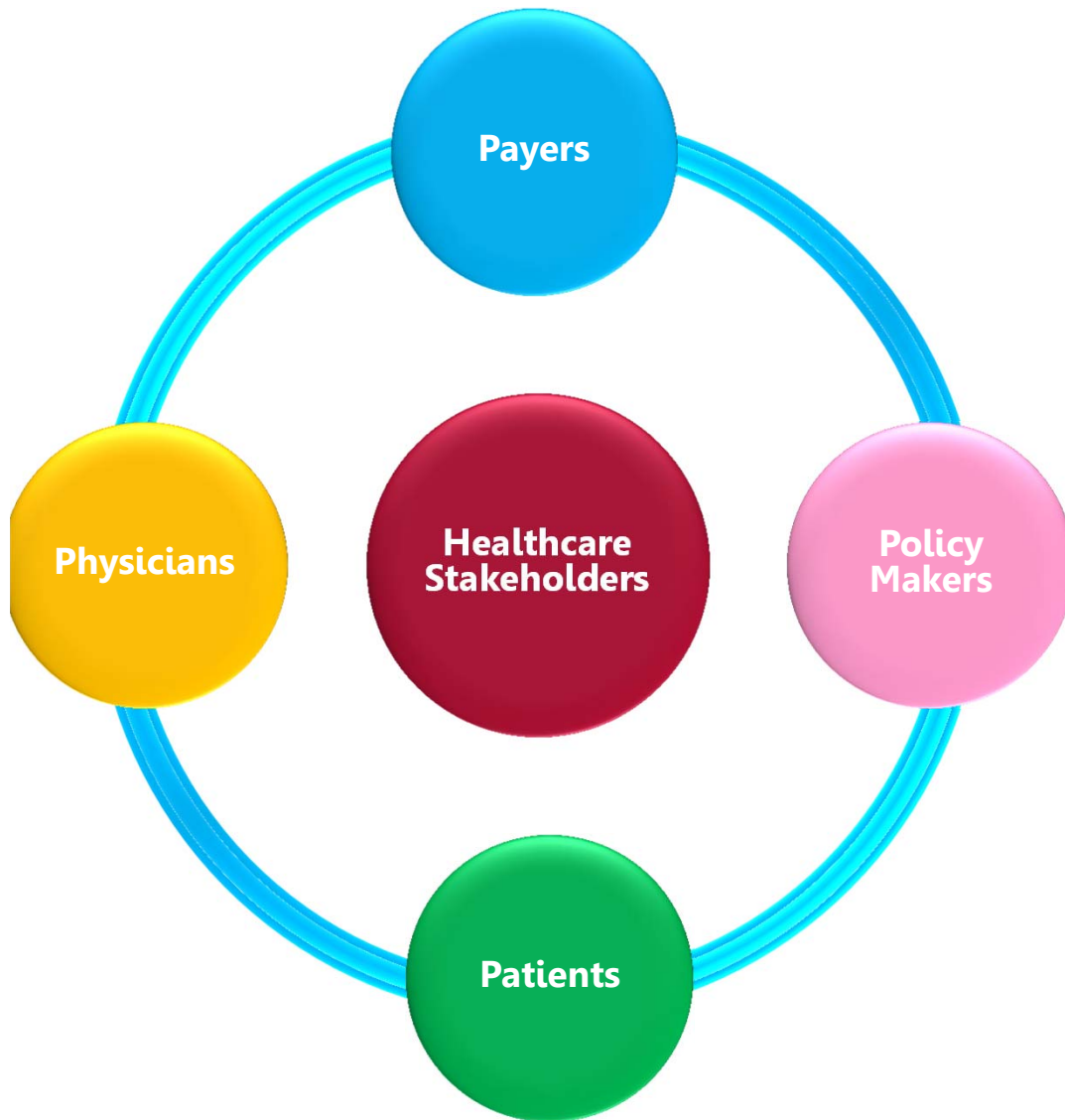


Source: IMS Health MIDAS, Dec 2014; The World Bank, 2015

Reference: A, B

- U.S. continues to be the leader in per capita spend (~\$100) in 2014
- Percentage increase in 5-year period has been highest in UK (67%)

Oncology treatment value is increasingly being questioned by patients, physicians, payers and policy makers



Patients

- Will this newer treatment improve my survival?
- Will this newer treatment improve my Quality of Life?
- Why is this treatment so expensive?
- Give me real-life examples?

Physicians

- Show us the performance of newer drug as an improvement in primary & secondary outcomes?
- Is this a cost-effective option?
- Can you show us the real-world evidence of the newer therapy?

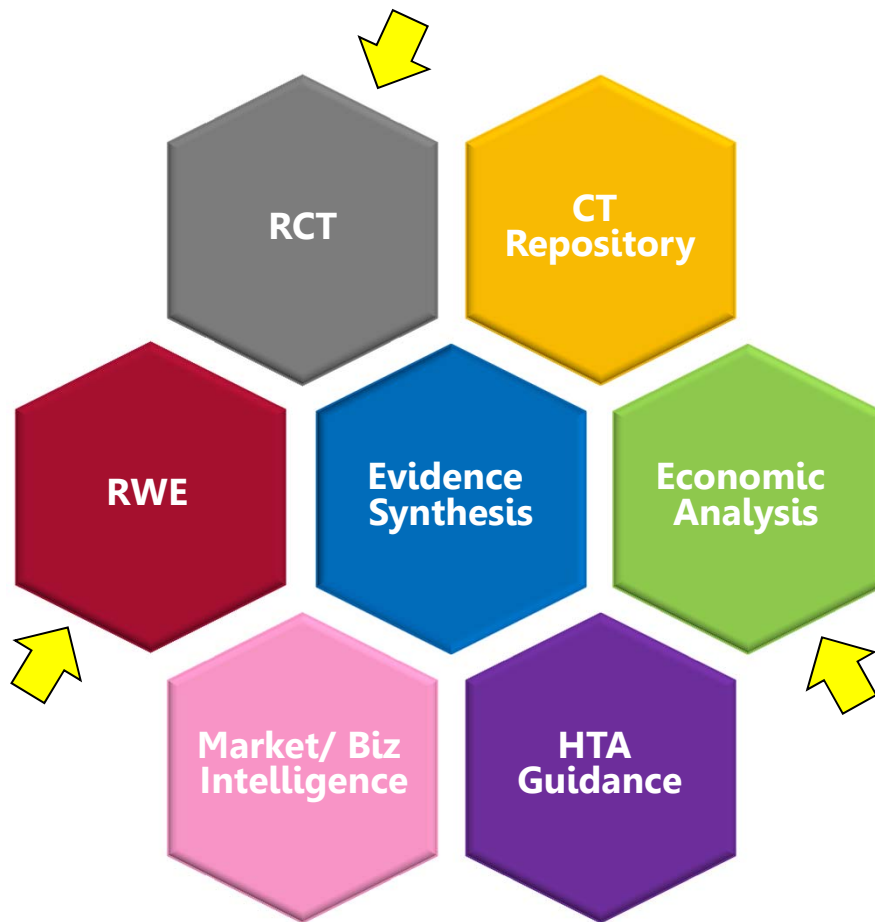
Payers

- Is this drug therapy needed?
- How safe and efficacious is this treatment?
- Is this a cost-effective option?
- Can you show us the Comparative or relative effectiveness?

Policy Makers

- Can this new treatment provide performance based pricing?
- Can you produce economic analyses?
- Is this treatment qualify for comparative effectiveness?
- Can you show us the real-world evidence?

To answer all these questions, it is imperative to gather evidence from multiple sources



Randomized Controlled Trials (RCTs)

- ✓ "Gold-standard" evidence

CT Repository

- ✓ Upcoming and ongoing trials & endpoints

Economic Analysis

- ✓ CEA, CUA, CBA, CMA, BIM etc.

HTA Guidance/ Appraisals

- ✓ Clinical, economic assessments

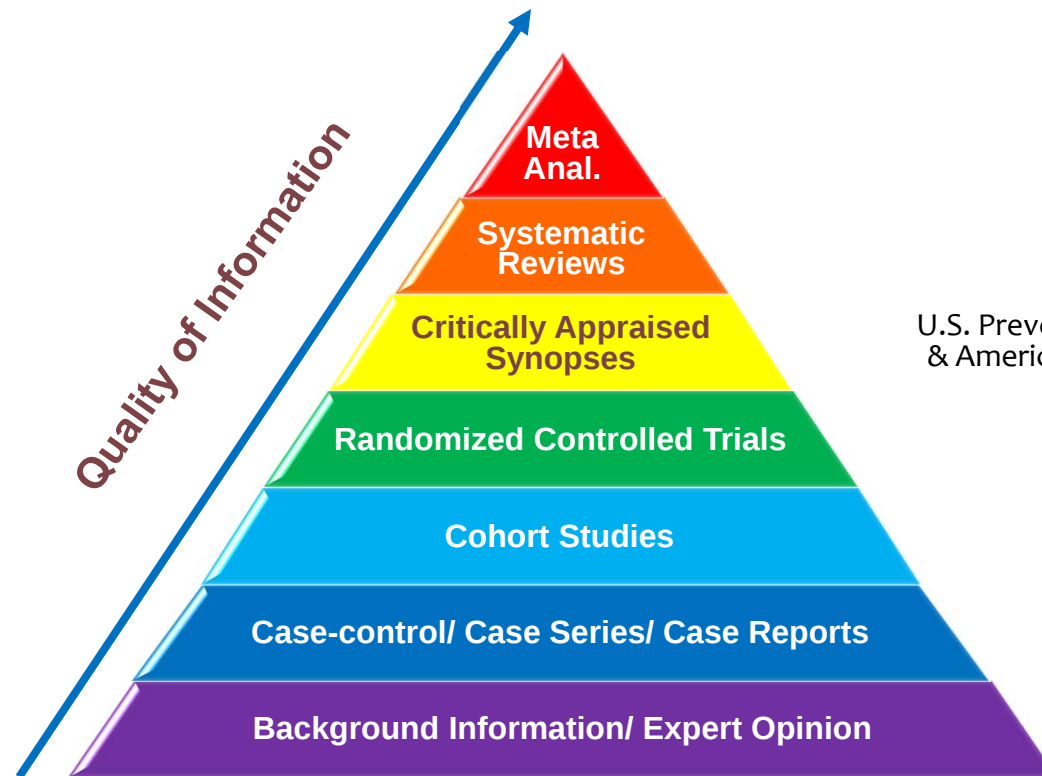
Market Dynamics/ Biz. Intelligence

- ✓ Development programs, competition etc.

Real-World Evidence

- ✓ Observational/ Registry/ EMR/ Claims/ PROs

Traditionally meta analysis of RCTs is considered as the “best” level of evidence



U.S. Preventive Services Task Force
& American College of Cardiology

Reference: C, D, E

- This hierarchy is designed to reflect relative internal validity, which is well suited to regulatory decisions and clinical guideline development
- But this approach is not well suited to the broader evidence needs of post regulatory decision making

Traditionally meta analysis of RCTs is considered as the “best” level of evidence

- Limitations of RCTs
 - Comparative effectiveness evaluation not followed in most of the RCTs
 - Controlled environment, stringent inclusion exclusion criteria
 - Confounding by crossover design
 - Open label trials & un-blinded
- To summarize, other pieces of evidence are crucial in decision making



Reference: F

Economic analyses

Cost-effectiveness Analysis (CEA)

- Costs are measured in monetary units while benefits in outcomes
- $ICER = \frac{\text{Cost of A} - \text{B}}{\text{Benefits of A} - \text{B}}$
- Lower the ratio, more the treatment is preferred, when resources are limited



Cost Minimization Analysis (CMA)

- To find the least expensive way of achieving desired health outcomes
- CMA is applicable when branded & generic medicines are compared



Cost Utility Analysis (CUA)

- QALY is the best known utility measure
- CUA is used to compare different Trts for different disorders (Onc. vs. Vac.)
- Trts with lower CU ratios are considered more efficient



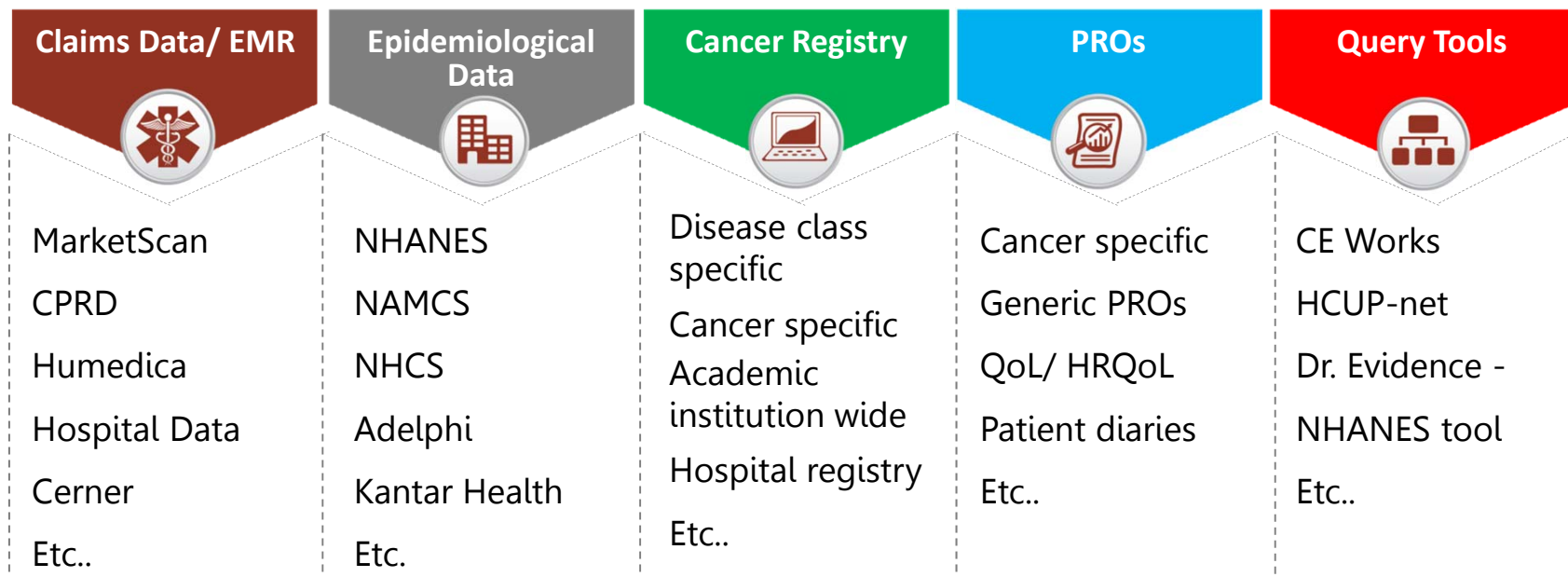
Cost Benefit Analysis (CBA)

- Both costs & benefits are given in monetary units (€, £, \$)
- Benefits are valued in monetary terms by placing values on individuals' observed or stated preferences
- Oldest form not commonly used in modern HE



RWE help answer various research questions for commercial and market access initiatives

- According to ISPOR Real World Data Task Force, "data used for decision making that are not collected in conventional RCTs are essential for sound coverage, payment, and reimbursement decisions."
- Various instruments of RWEs



RWE help answer various research questions for commercial and market access initiatives

- Various research questions are answered by RWE studies



Treatment Patterns & Trends



Burden/ Cost of Illness



Comparative Effectiveness



Safety Evaluations



Health Policy Analysis



Physician Practice Patterns



Medication Adherence

References

- A. IMS Health MIDAS, Dec 2014
- B. The World Bank, 2015
- C. Sackett, D., Rosenberg, W., Muir Gray, J., et al. (1996). British Medical Journal, 312, 71-72.
- D. U.S. Department of Health and Human Services, Agency for Health Care Policy and Research. Acute Pain Management: Operative or Medical Procedures and Trauma. Clinical Practical Guideline No. 1. Publication no.92-0023, 107. Rockville, MD: AHCPR, 1993.
- E. American College of Cardiology Foundation. Clinical Statements/Guidelines, 2006. Available at <http://www.acc.org/qualityandscience/clinical/statements.htm>, accessed June 29, 2009.
- F. Mullins D.; Uncertainty in Assessing Value of Oncology Treatments; The Oncologist 2010;15 (suppl 1): 58-64
- G. Health Economics, 5/E, Charles E. Phelps; ISBN-10: 0132948532; Published 05/29/2012

Thank You!

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