Β΄ Περίοδος/ 18η Συνάντηση
«Ποσοτικές μετρήσεις και ποιοτικές προσεγγίσεις στην υγεία και την ιατρική περίθαλψη»
Ξενοδοχείο Hydrama, Δράμα
28-30 Σεπτεμβρίου 2018

«Καταγραφές δεδομένων και Μητρώα ασθενών: μια προσέγγιση για τη βέλτιστη διαχείριση των νοσημάτων»

Έλλη Βίτσου
Health & Value Lead, Pfizer Hellas

Pressures on the healthcare system are changing the conversation, "Is the Cost worth the Value?"

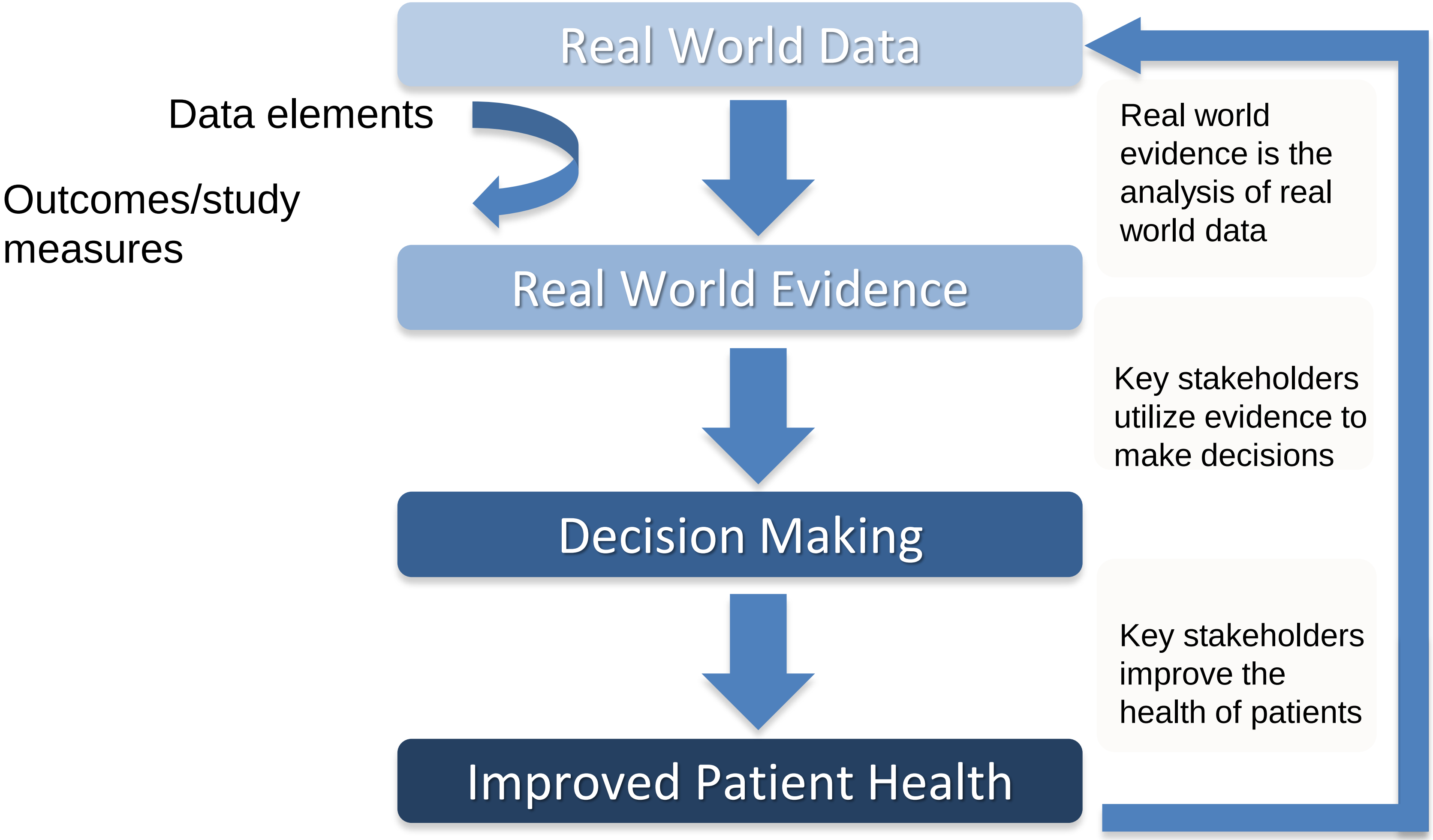
Key Challenges

- Aging population
- Greater prevalence of chronic diseases
- Expensive specialty medications
- Researchers targeting more complex diseases
- General economy

Impact

- Healthcare spending
- Premiums
- Budget constraints
  - Access restrictions
  - Hurdles for demonstrating value and securing reimbursement are increasing

Analysis of RWD generates RWE to inform and improve patient health



Real World Data

Data elements

Outcomes/study measures

Real world evidence is the analysis of real world data

Real World Evidence

Key stakeholders utilize evidence to make decisions

Decision Making

Key stakeholders improve the health of patients

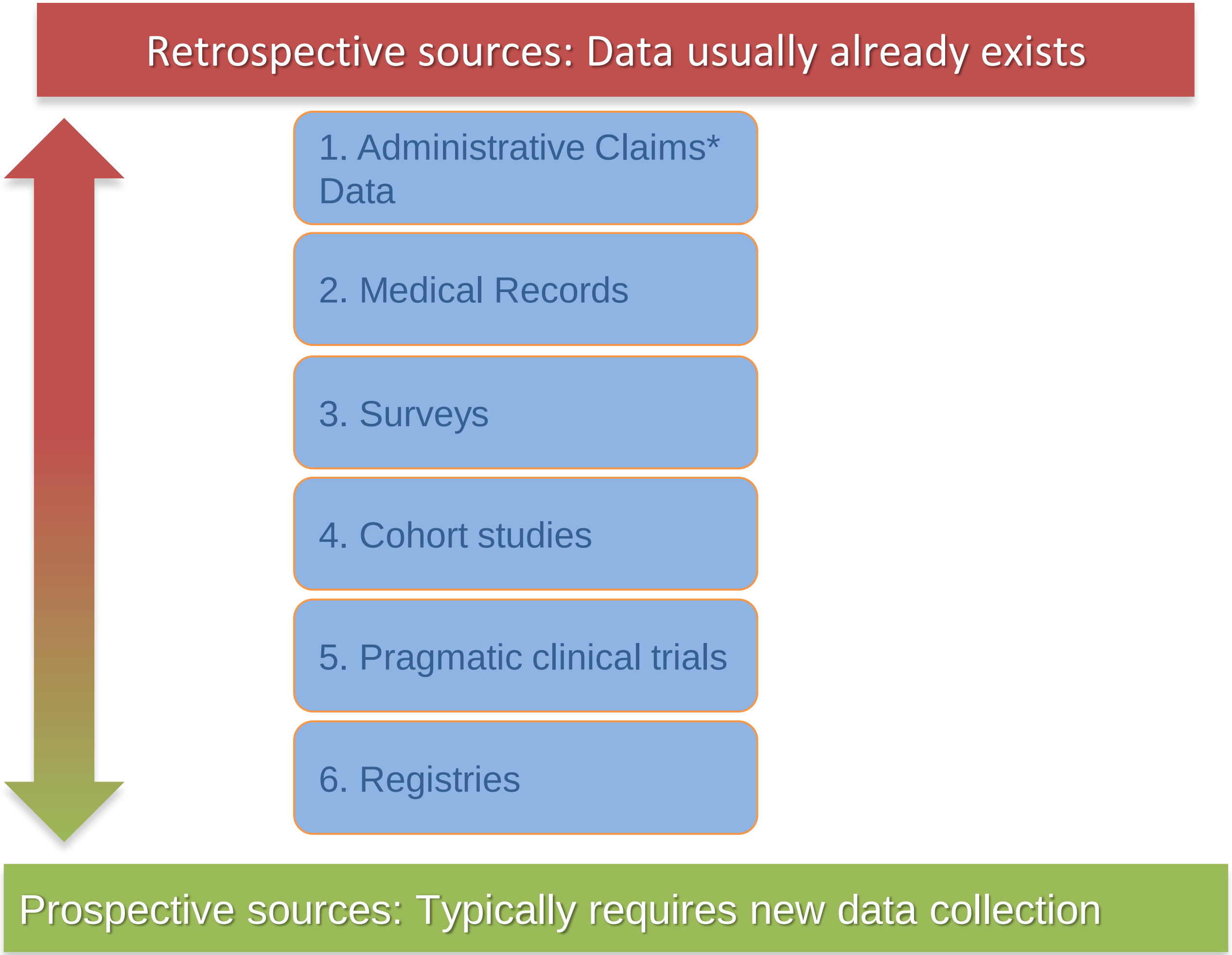
Improved Patient Health

Source: Garrison, LP, et al. Value in Health. 10(7):326-335

RWE offers insights beyond the “gold standard” RCT

Real World Evidence	Clinical Trial Data
• Prospective and/or retrospective data collection	• Prospective data collection
• Real-world patient adherence and compliance	• Always good patient adherence and compliance
• Focus on real-world use and practice (potential for switching, discontinuing and/or adjunctive meds)	• Focus on drug efficacy and side effects
• Inexpensive relative to clinical trials and faster to execute	• Very expensive to conduct and takes a long time
• Expanded generalizability	• Limited generalizability—segment of the population is eligible for inclusion
• High ability to evaluate medical costs and value within local health economy	• Limited ability to evaluate medical costs

There are many types of RWD – both retrospective and prospective



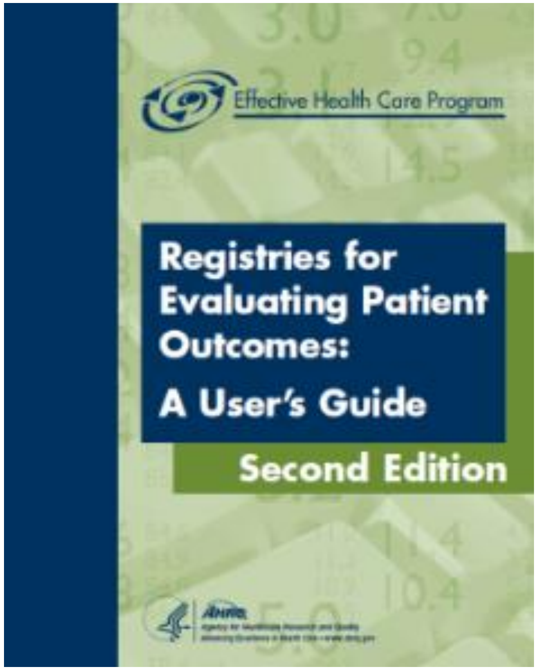
Retrospective sources: Data usually already exists

1. Administrative Claims* Data
2. Medical Records
3. Surveys
4. Cohort studies
5. Pragmatic clinical trials
6. Registries

Prospective sources: Typically requires new data collection

**Claims refers to the paper trail of collecting reimbursement from health insurance funds/companies for services rendered by providers*

Patient Registries provide RWD that complement clinical trial evidence



Effective Health Care Program

Registries for Evaluating Patient Outcomes: A User's Guide

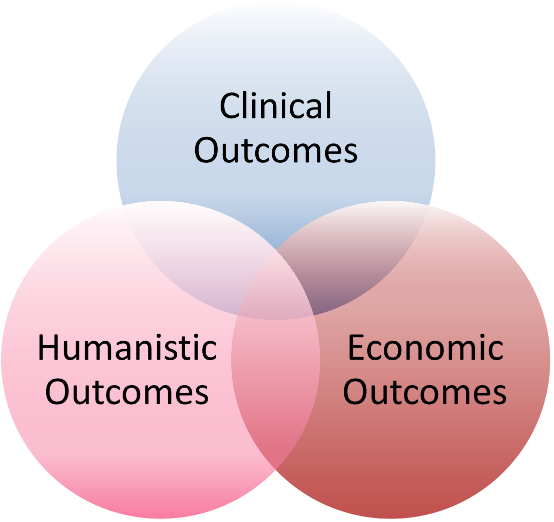
Second Edition

A patient registry:

- Is an organized system that uses observational study methods to collect uniform data (clinical and other)
- Evaluates specified outcomes for a population defined by a particular disease, condition, or exposure, and that
- **Serves a predetermined scientific, clinical, or policy purpose**

Gliklich RE et al. Registries for Evaluating Patient Registries: A User's Guide: AHRQ publication No. 07-EHC001. Rockville, MD. April 2007

Data elements and outcomes/measures associated with registry data



DATA ELEMENTS

Humanistic

- Sex
- Age
- Race/ethnicity
- Geographic region
- Sociodemographics
- HRQoL, health status, symptoms, functioning, patient satisfaction with care, patient preferences

Clinical

- Diagnosis code (ICD-9, ICD-10)
- Procedure code (ICD-9, CPT-4, HCPCS)
- Dates of service, admission & discharge dates
- Treatment course (drug name, route, dose, response, reason for drug behavior)
- Vitals, laboratory values, risk factors (smoking, alcohol use)
- Mortality (including cause of death)

Economics

- Dates of service, place of service
- Indirect costs (work productivity, work loss, caregiver-related, transportation)

Sources: 1) AMCP Outcomes Research 2015; 2) AHRQ Outcome Definition and Measurement

Complexity and quality varies between different RWD sources

	Description of data source	Resource use		Level of complexity	Quality of evidence
		Personnel	Finance		
Administrative claims data	Databases of pharmacy/medical care use and associated payment information collected in the process of adjudicating claims/payments by payers		$	↑↑	+++
Chart review/ electronic medical records	Databases of prospective healthcare provider-captured clinical notes and patient health records from routine care and follow-up		$$	↑↑	+++
Patient survey/PROs	Direct assessment of patient experience, unmet needs and PROs in real-world practice settings		$$	↑↑↑	+++
Registries	Non-interventional or post-interventional follow-up of patients to assess effectiveness of products over time in a real-world/usual practice setting		**$$$**	**↑↑↑↑**	**++++**

Measures represent what may be considered typical

- Registries are typically expensive to initiate, manage and maintain
- Evidence from registry data may be of high quality
- Data platforms underpinning registries may have high level of sophistication

Clinical audits and registries in UK

- Clinical audits[1] and registries work collaboratively with clinical specialists, patient representatives and universities
- NHS Digital is responsible for collecting, transporting, storing, analysing and disseminating the nation's health and social care data.
- The NHS Digital data and information strategy, aims to **enable the health and care system to provide the best possible services and to achieve world class health outcomes.**

1. https://digital.nhs.uk/data-and-information/clinical-audits-and-registries

National Cancer Registration and Analysis Service (NCRAS)

How cancer registration data is used

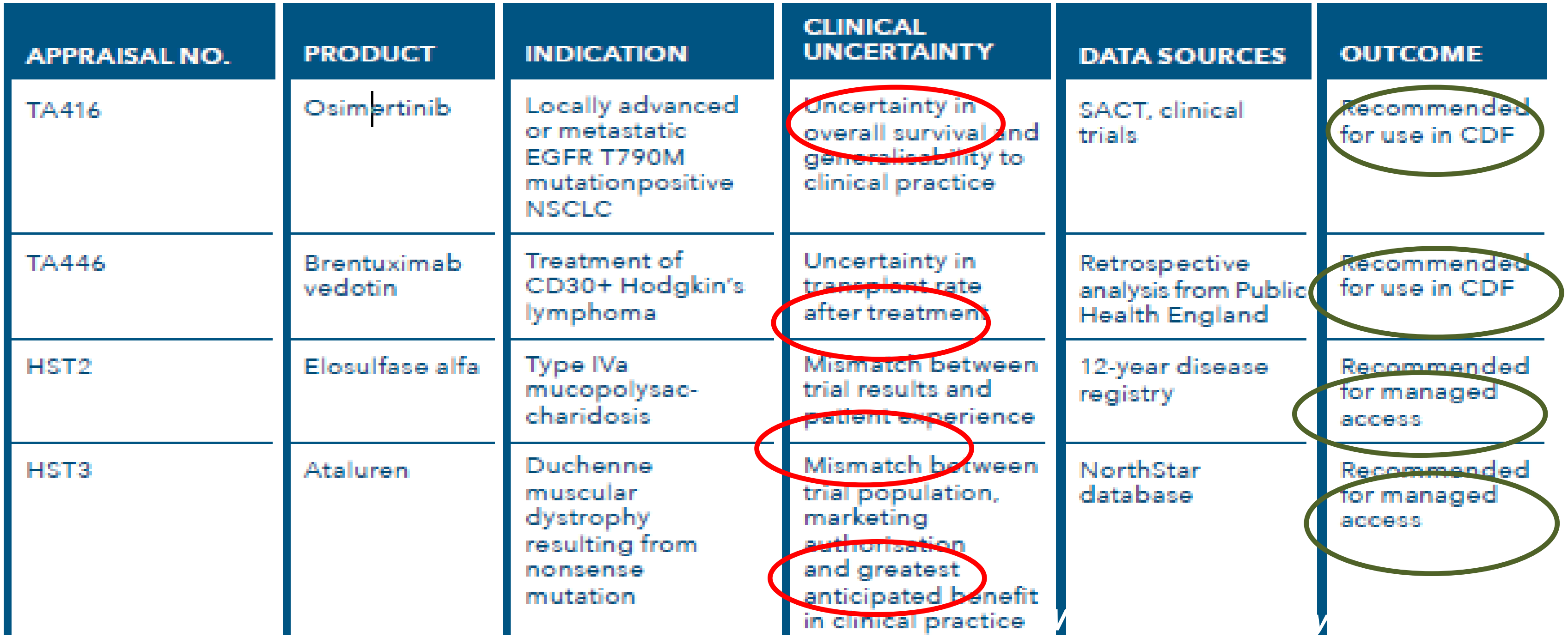
- **Monitoring cancer rates:** Cancer registration is the only way we can keep track of ***how many people are getting cancer and what types of cancer they have***. It tells us how many people are ***diagnosed with cancer each year***, how many people are living with cancer at any one time, and the ***survival outcomes*** for people with different types of cancer.

- **Improving cancer care:** Cancer registration data is vital to ensure the NHS can ***follow up*** the long term effects of a treatment that may have been given many years earlier. Cancer registration data enabled the NHS to contact women treated with radiation for Hodgkin’s disease (a cancer of the lymphatic system) when they were younger because they might have an increased risk of breast cancer. The treatments for Hodgkin’s disease have been changed as a result.

Using RWE to inform NICE technology appraisals

RWE has an important role in addressing clinical uncertainties for drugs approved in managed access schemes via the CDF[1]

Table 2. RWE has been used successfully to inform NICE HTAs



APPRAISAL NO.	PRODUCT	INDICATION	CLINICAL UNCERTAINTY	DATA SOURCES	OUTCOME
TA416	Osimertinib	Locally advanced or metastatic EGFR T790M mutationpositive NSCLC	Uncertainty in overall survival and generalisability to clinical practice	SACT, clinical trials	Recommended for use in CDF
TA446	Brentuximab vedotin	Treatment of CD30+ Hodgkin's lymphoma	Uncertainty in transplant rate after treatment	Retrospective analysis from Public Health England	Recommended for use in CDF
HST2	Elosulfase alfa	Type IVa mucopolysaccharidosis	Mismatch between trial results and patient experience	12-year disease registry	Recommended for managed access
HST3	Ataluren	Duchenne muscular dystrophy resulting from nonsense mutation	Mismatch between trial population, marketing authorisation and greatest anticipated benefit in clinical practice	NorthStar database	Recommended for managed access

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1. *Insights from a conference co-hosted with the London School of Economics, **REAL-WORLD EVIDENCE IN ONCOLOGY,** Towards Innovative and Affordable Patient Care*
2. *RWE AND OUTCOMES-BASED COMMISSIONING IN ONCOLOGY, P.Huskinson National Commercial Director, Specialised Commissioning, at NHS England*

Belgian Cancer Registry

Belgian Cancer Registry

- Collects information about all new cancer diagnoses in Belgium and their follow-up. Based on this information it maps out the nature and extent of cancer in Belgium
- ***Evidence based guidelines*** are developed and published in collaboration with the Belgian Health Care Knowledge Centre
- ***Quality indicators*** derive from the guidelines and used to create feedback on provided services
- Use of registry data in Belgium could be included in ***pharmacoeconomic models*** and support the discussion with payers

http://www.kankerregister.org/Background

1. **Real-world evidence What role can it play in real-world decision-making? Summary report of the IHE Roundtable December 12, 2016**

Collaborations between registries

ECTRIMS
EUROPEAN COMMITTEE FOR TREATMENT AND RESEARCH IN MULTIPLE SCLEROSIS

Big Multiple Sclerosis Data network: data sharing among five large MS registries

The BMSD network will allow pooling of MS data at a scale that ***raises MS research to a new level, with the ultimate aim to lessen in the future the burden of the disease for MS patients.*** BMSD will overcome major unmet scientific needs in MS requiring large long-term longitudinal data.

European Network of Cancer Registries

The European Network of Cancer Registries (ENCR) promotes collaboration between population-based cancer registries, with coordinating activities and **mapping of priorities for research topics** and regularly disseminates information on **incidence and mortality from cancer in the European Union and Europe**.

INPDR

What is the INPDR: The INPDR is a collaboration between clinicians, scientists, researchers and patient associations across the world to collect clinical, genetic, diagnostic and outcome data from patients with Niemann-Pick Diseases (NPD).

Καταγραφές δεδομένων στην Ελλάδα

Μητρώα ασθενών

- Ηπατίτιδα C (επανέναρξη λειτουργίας)
- Χρόνια Μυελογενή Λευχαιμία
- Μητρώο Περιτοναϊκής Κάθαρση
- Μητρώου Απομυελινωτικών Παθήσεων του ΚΝΣ (υπό κατασκευή)
- Σακχαρώδη Διαβήτη (υπό κατασκευή)
- Συνενζύμου Q10 (υπό κατασκευή)

Using Big Data to Assess Prescribing Patterns in Greece: The Case of Chronic Obstructive Pulmonary Disease

Kyriakos Souliotis, Chara Kani, Manto Papageorgiou, Dimitrios Lionis, Konstantinos Gourgoulianis

Published: May 18, 2016 • https://doi.org/10.1371/journal.pone.0154960

○ Συμμετοχή όλων των εμπλεκόμενων (Ιατρικές Εταιρείες, Ενώσεις και Σύλλογοι Ασθενών, Εποπτευόμενοι Φορείς του Υπουργείου Υγείας) είναι βασική προϋπόθεση για την επιτυχή σχεδίαση και ολοκλήρωση

Better data, better decisions, better health outcomes