Ανάπτυξη και εφαρμογή προγραμμάτων RWD. Ο ρόλος των Ιατρικών Τμημάτων ΦΕ.

Μάνος Κουταλάς, MD

Ειδικός Παθολόγος - Λοιμωξιολόγος, MAF Mgr

janssen
PHARMACEUTICAL COMPANIES
of Johnson & Johnson

What is Real World Data (RWD);

> "Real World Data" are observations of effects based on what happens after a prescriptive (treatment) decision is made where the researcher does not or cannot control who gets what treatment and does not or cannot control the medical management of the patient beyond observing outcomes

– ISPOR task force

Real World Evidence/Data

- Everything that goes beyond what is normally collected in the phase III clinical trials program

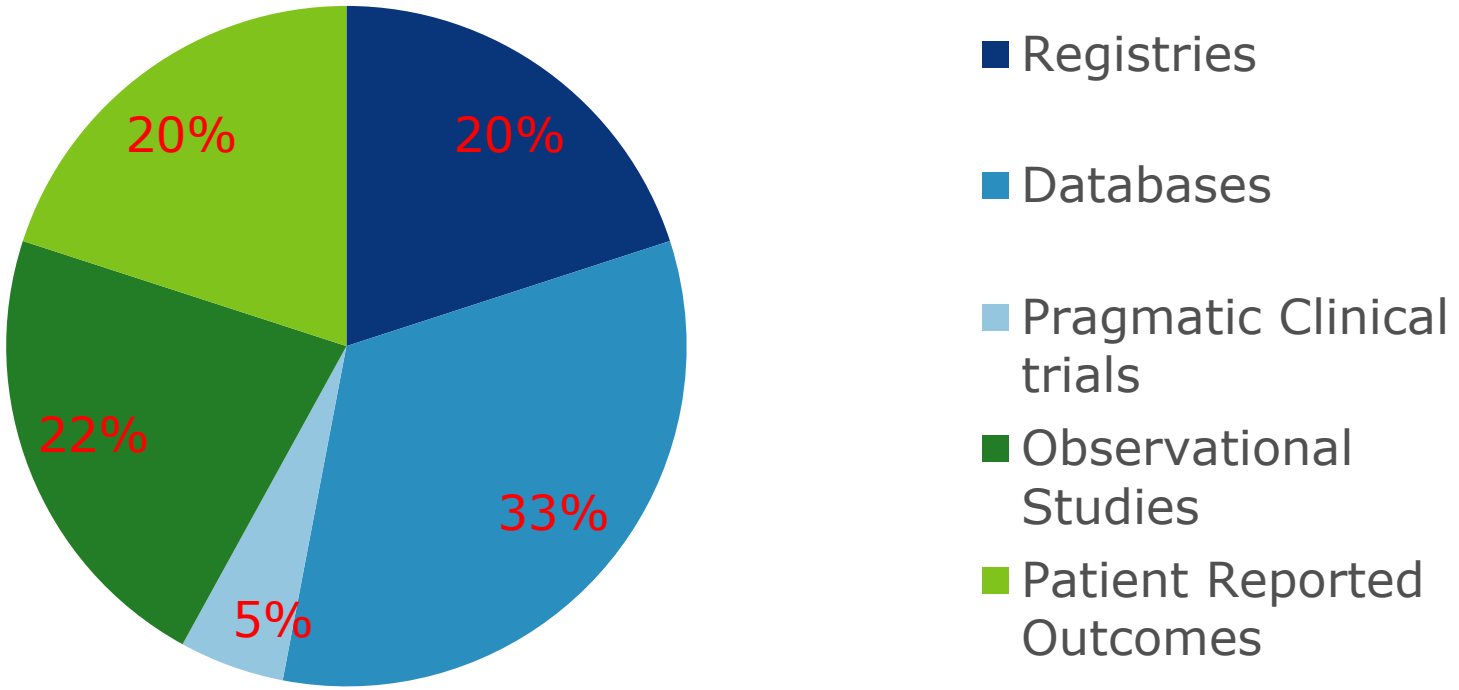
Importance of RWD

- Improving health outcome (better interpretation of clinical data)
- Offering insights into what happens in everyday clinical care
- Comparing multiple alternative clinical strategies

Changing pharma business model - going forward

(Reducing costs-Developing future clinical trial design-Reimbursement)

Sources of RWD



janssen | PHARMACEUTICAL COMPANIES OF Johnson & Johnson

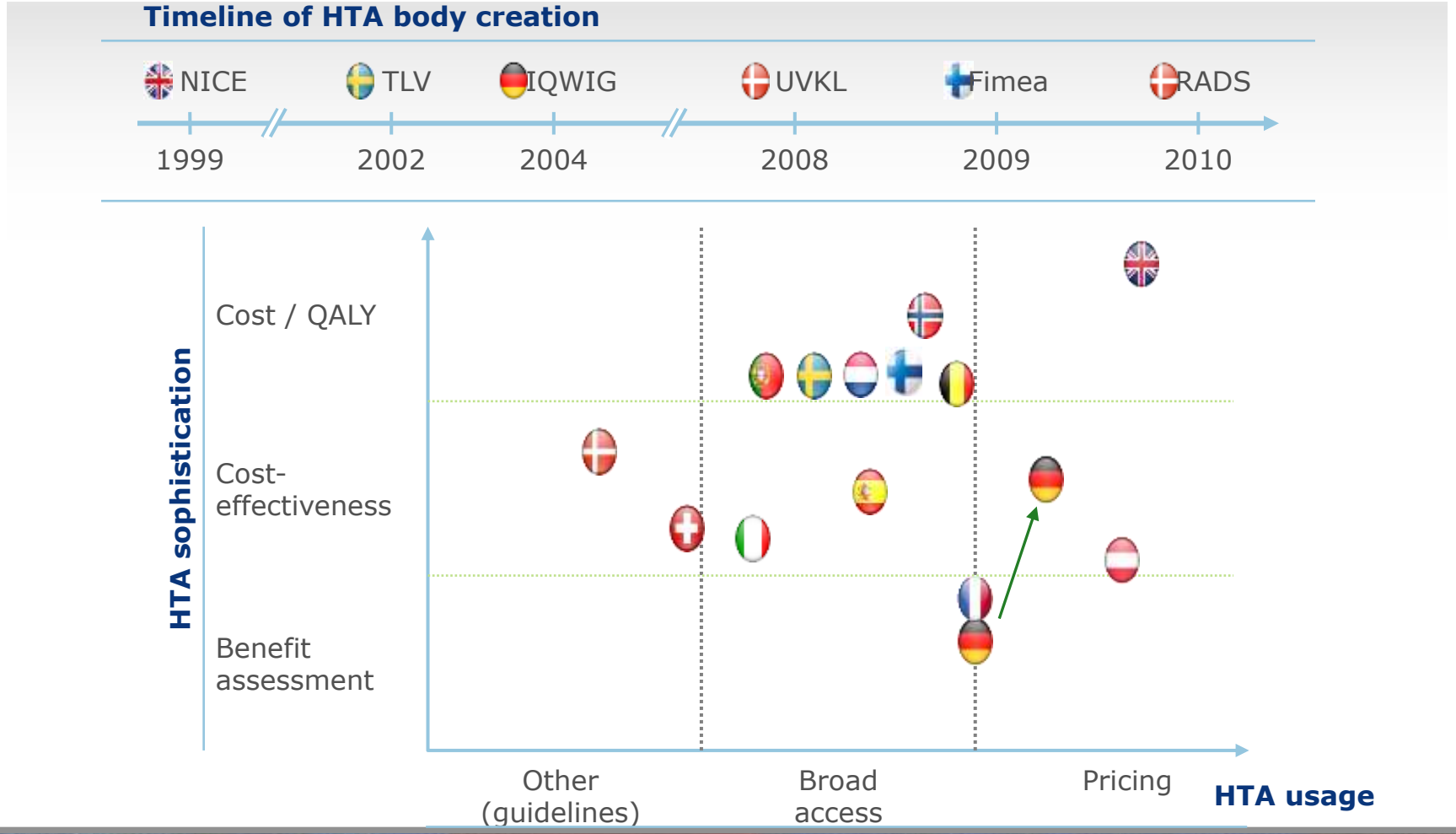
Source: eyeforpharma-Real World Data-Sources & Applications

- A **patient-reported outcome or PRO** is a questionnaire used in a clinical trial or a clinical setting, where the responses are collected directly from the patient.
  - Examples: Health status, General health perceptions, Quality of life (QoL) questionaires , etc
- **Pragmatic trials** measure *effectiveness*-the benefit the treatment produces in routine clinical practice.
  - Example: two physiotherapy approaches are being evaluated for back pain. The protocol may allow for the physiotherapist to apply different treatments to different patients: it is then the management protocol which is the subject of the investigation, not the individual treatments.

5 key elements in an Evidence Generation project

	Experimental	Real World
Experimental vs Real World		
Interventional vs Non-interventional	RCT is the gold standard to measure efficacy	RWD is the gold standard to measure effectiveness/efficiency
Prospective vs Retrospective	⊕ Highly selected sample	⊕ Strong External validity
Primary vs Secondary data	⊕ Selection and Measure bias well controlled	⊕ Cost
Internal vs External Data Source	⊖ External validity	⊖ Variability
	⊖ Cost	⊖ Selection & measure bias
	⊖ Time	

There is increasing health system demand for pharmacos to demonstrate and improve "outcomes"



SOURCE: Expert interviews; McKinsey

"Outcomes": Definition

***Our definition of "outcomes":**

- Improving **primary health endpoints** in a real world setting (e.g., reduced mortality)
- Improving **secondary health endpoints** (e.g., reducing comorbidities, enhancing quality of life)
- **Improving system cost** to achieve existing endpoints (e.g., reducing cost to treat side effects associated with current treatments)

Why do we care about RWD in context of "outcomes"?

Increasing importance

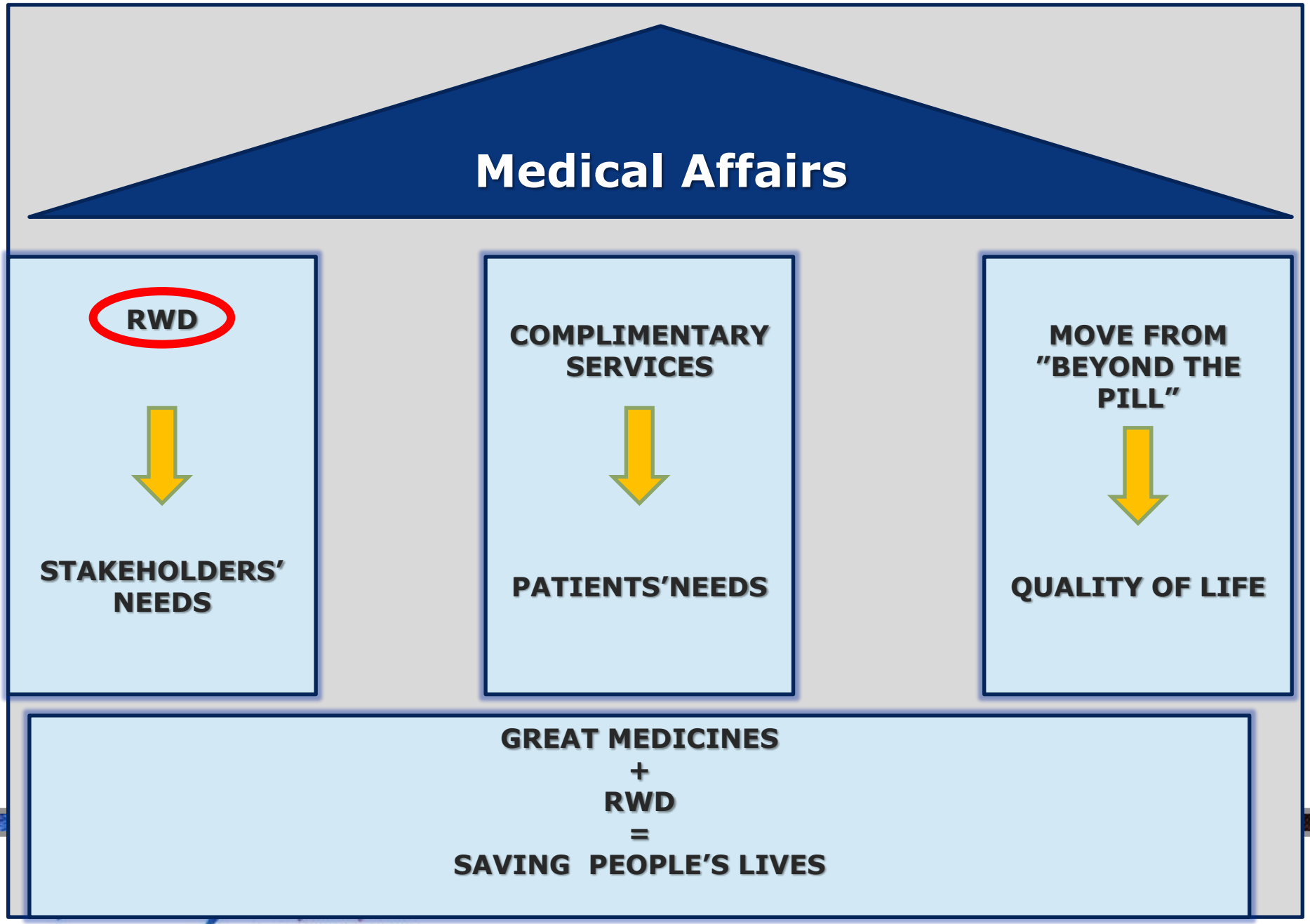
1. Payors and regulators are asking for it
2. Payors are using it
3. Increased infrastructure (databases) changing the rules

Opportunity & threat

4. Decisions across lifecycle
5. Transparency & control on product information
6. Competitors emerging; success / proactive use

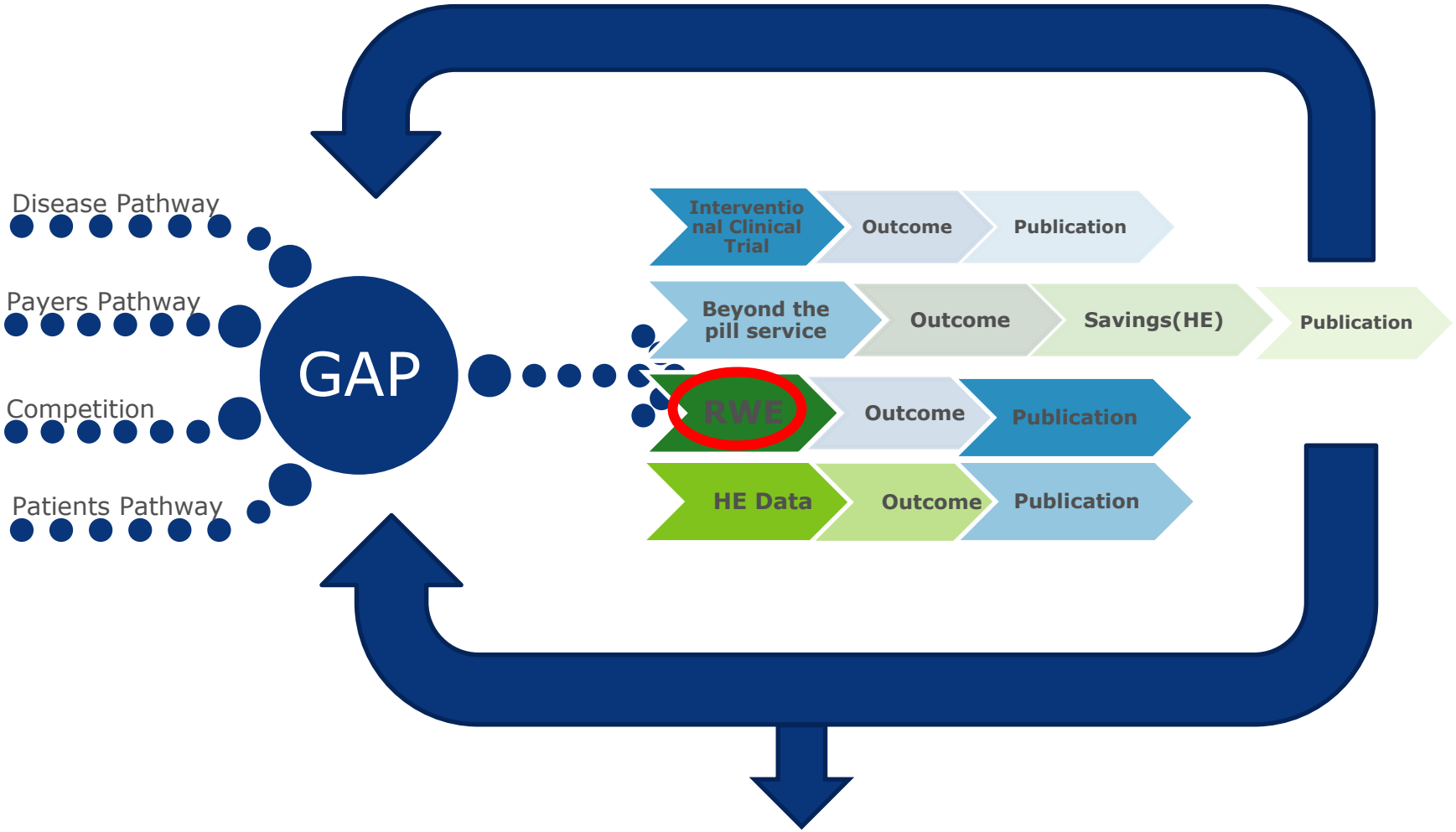
Requirements for effective use

7. Systematic RWD planning (early evaluation of options)
8. Addressing ongoing barriers (technical & organisational)



Modified from Boston consulting Group: Competing on Outcomes (1/2014)

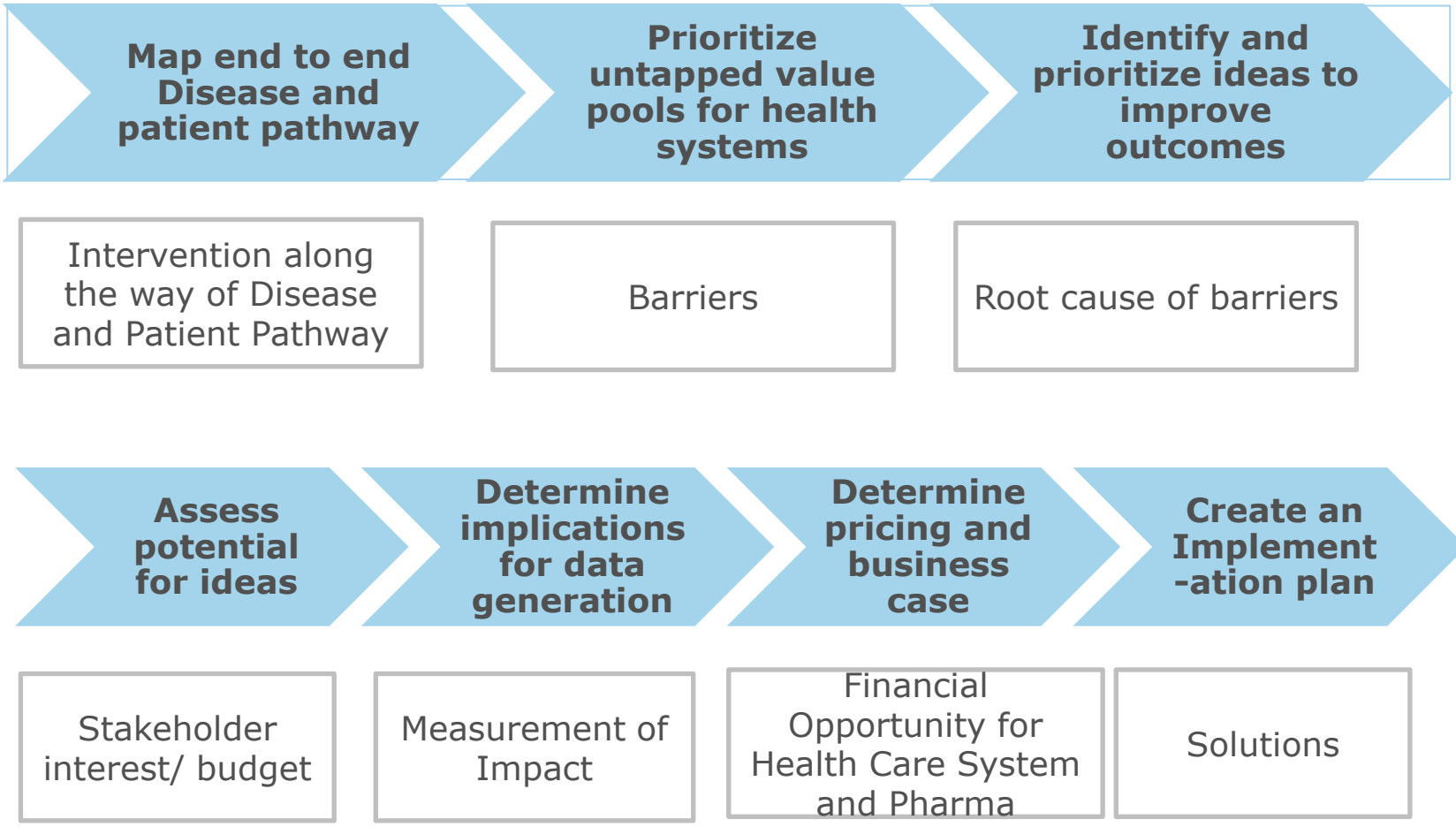
A non stop cycle for MAF



janssen | PHARMACEUTICAL COMPANIES OF Johnson&Johnson

Continuous Improvement on Patient's Lives

Methodology for Outcomes/RWD



Where only RWD can do the job

- Measuring (comparative) Effectiveness
- Analysing treatment patterns and drug utilization
- Understand Disease management strategies
- Long-term benefits or harm
- Surveillance of rare events / rare treatment combinations
- Rare diseases / Orphan diseases

RWD READY ?

Real World Evidence challenges

- Not unified regulatory landscape (vs EU-CTD)
- Designing projects is more complex
- Preparing and conducting analysis require specific expertise
- Acceptability of RWE by external bodies / scientific community (TRUST)
- Competition generating negative RWE
- Co-partnering strategy
- Other stakeholders (regulators, payors, scientific societies) also generating their data

RWD: what is in place

- Some experience & expertise already exist in:
  - Medical Affairs with Company-sponsored non-interventional projects
  - MAF/HE with internal / external databases
  - Business Intelligence with external data / interviews
- Organise & develop our RWE capabilities & capacities

MAF next steps

BACKBONE

Internal

- **Change management Plan**

External

- **Environment Scan:** What is the future governance model

"WHAT" TO PREPARE

- **Internal Mapping of Processes**
- **Analysis to address stakeholders RWD needs**

- **A "*how to..*" Guide**

"HOW" TO IMPLEMENT

- **Roles & Responsibilities**
- **Resources**
- **Additional Future Capabilities**

Key takeaways - Conclusions

- MAF has a dominant role for RWD creation
- Analysis of local environments/stakeholders needs, necessary
- New roles and responsibilities for MAF personnel

The journey begins!

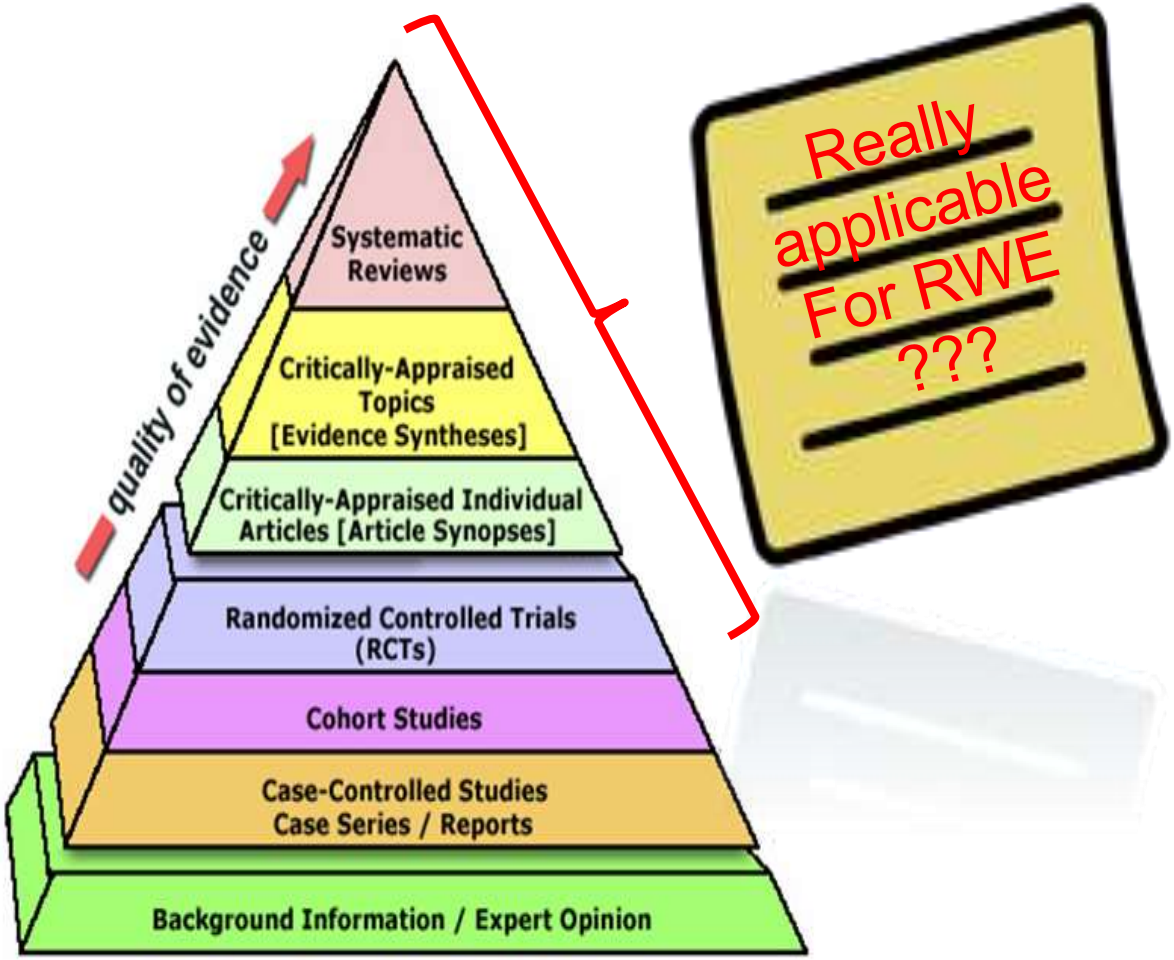
Backup slides

WHEN SPEAKING ABOUT REAL WORLD DATA/EVIDENCE, PEOPLE OFTEN FEEL LIKE…


BILL MURRAY SCARLETT JOHANSSON
Lost In Translation

janssen | PHARMACEUTICAL COMPANIES OF Johnson & Johnson

Traditional hierarchy of evidence for experimental research



5 key elements in an Evidence Generation project

Interventional	Non-interventional
Pragmatic clinical trials • Intervention at start • But patients are then followed as in routine	
⊕ Selection & measure bias ⊕ Comparative analysis	⊕ Cost ⊕ Administrative burden
⊖ Cost ⊖ Administrative burden (EU-CTD applies)	⊖ Data variability ⊖ Data quality (site qualification)

5 key elements in an Evidence Generation project

- ***Experimental vs Real World***
- ***Interventional vs Non-interventional***
- ***Prospective vs Retrospective***
- ***Primary vs Secondary data***
- ***Internal vs External Data Source***

	Interventional	Non-Interventional
Experimental Settings	Explanatory Randomized Trials	
Real World Settings	Pragmatic Randomized Trials	Retrospective & Prospective Observational Studies, including cohort and case-control designs

5 key elements in an Evidence Generation project

Prospective	Retrospective
Data collection starts before event takes place	Data collection starts after event takes place
⊕ Robustness	⊕ Cost
⊕ Selection & measure bias	⊕ Time
⊕ Comparative analysis	⊕ Size of sample
⊖ Cost	⊖ Comparative analysis
⊖ Time	⊖ Selection & Measure bias

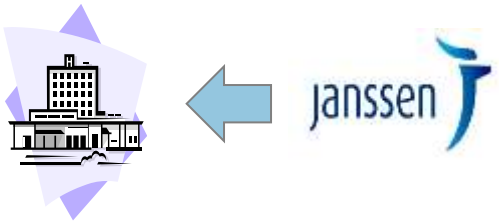
5 key elements in an Evidence Generation project

Primary Data	Secondary Data
Data generated for a specific research question	Data were originally not generated to answer the research question
⊕ Robustness	⊕ Cost
⊕ Selection & measure bias	⊕ Time
⊕ External validity	⊖ Re-analysis
⊖ Cost	⊖ Selection bias
⊖ Time	

5 key elements in an Evidence Generation project

- ***Experimental vs Real World***
- ***Interventional vs Non-interventional***
- ***Prospective vs Retrospective***
- ***Primary vs Secondary data***
- ***Internal vs External Data Source***

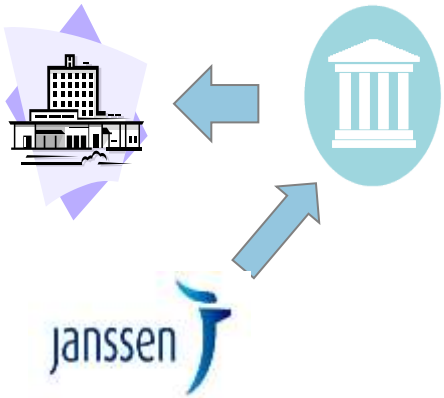
Internal Data



Data generated by our company acting as a sponsor of the Data Generation activity (directly or via CRO)

- Primary data from Janssen
- Other Janssen data

External Data



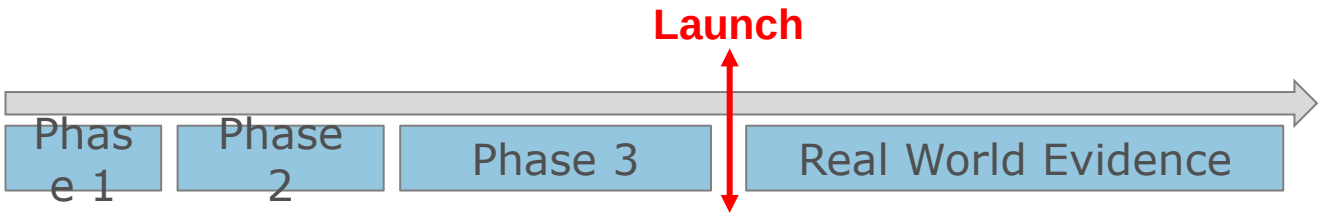
Janssen negotiate access to Data generated by another group (Janssen is not the sponsor)

- Patient records
- Insurance databases
- Scientific societies registries

Real World Evidence as part of Evidence Generation

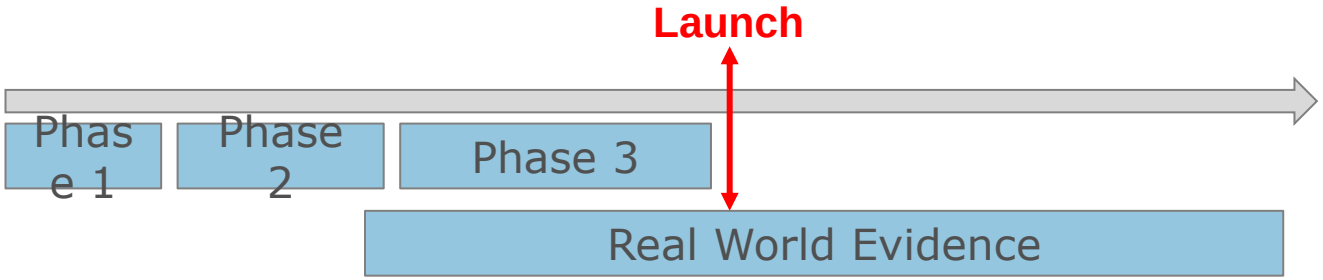
CURRENT APPROACH:

- Mainly post-launch
- Reactive



IDEAL APPROACH:

- Initiated pre-launch
- Proactive & reactive



RWE workstream process map

1 Phase I Concept design and prioritisation	2 Phase II Protocol writing, design and contracting	3 Phase III Study execution and reporting
 Align and derive strategic TA impli-cations for evi-dence generation	 Systematically define data strategy options	 Develop imple-mentation plan and milestones
 Perform data gap analysis	 Assess and select specific sources and partners	 Execute study or manage CRO to execute the study
 Develop research questions/stud y ideas	 Develop study concept & derive process implication	 Perform study analysis
 Scan available data sources	 Decide on which options and how to	 Interpret analysis and generate results
 Develop study outline	 Develop and execute complete contracts	 Track execution progress
 Prioritize study outline taking into account OpCos	 Develop protocols and SAP	 Discuss and report study results

The journey begins!