POST-AUTHORISATION STUDIES IN EUROPE: BRIDGING THE GAP BETWEEN REGULATORY AND HEALTH TECHNOLOGY ASSESSMENT (HTA) STAKEHOLDER NEEDS BY BETTER DESIGN OF STUDIES

Massoud Toussi

Epidemiology, Europe

Real World Evidence Solutions

To begin from the beginning…

900 BC 800 AC 1900s 1970s

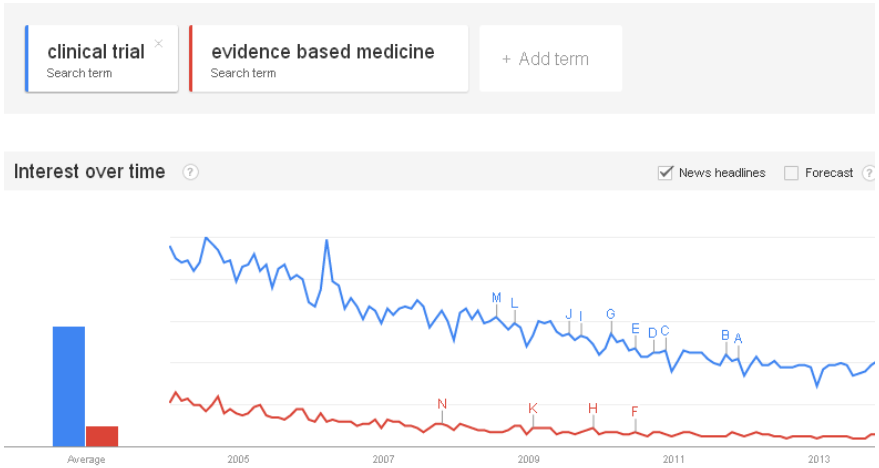
Trend changes

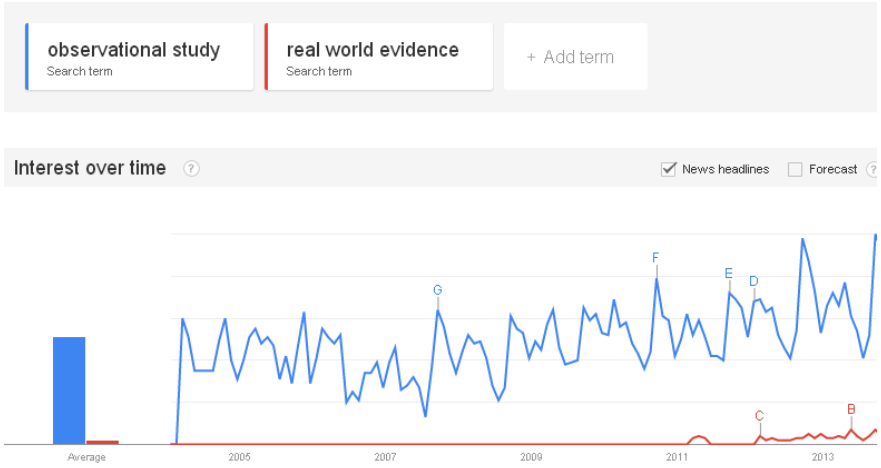
Disease oriented care

- EBM
- Clinical trials

Patient oriented care

- RWE
- Observational studies





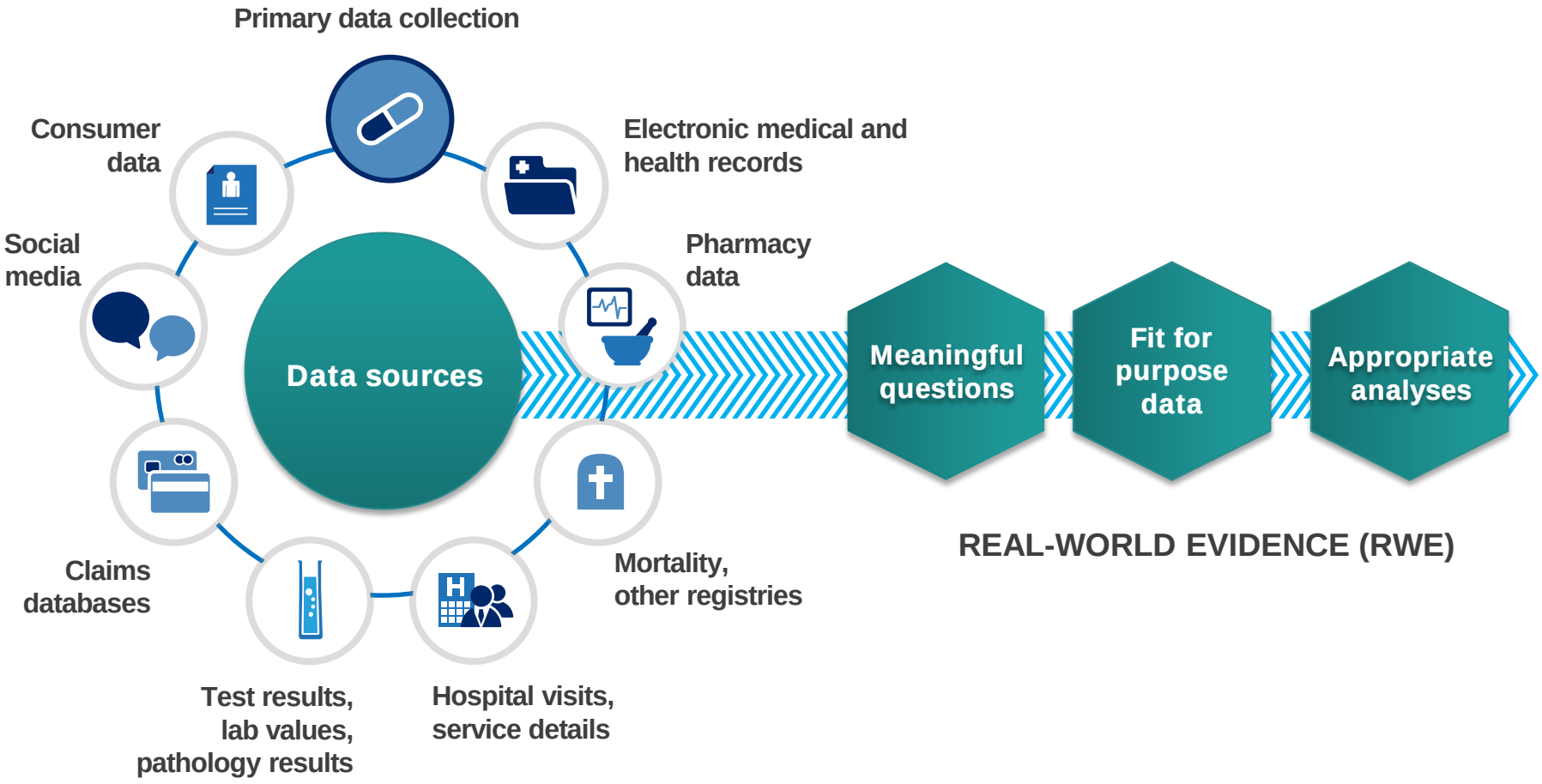
Real world studies: need for harmonization

	Clinical trials	Real world studies	
Research question, goals and design	Predefined goals expressed in 3 sequental phases Predefined endpoints	Research questions differ between regulatory and HTA bodies	
Ethical consdierations	Informed consent, IRB/IEC review	Different requirements for informed consent and IRB/IEC	
Good practice	ICH GCP	EMA GVP, ISPE GPP, GRACE, ISPOR checklist for databas studies, guidelines for database selection…	
Obligation for Registration	Generally Yes	Genrally No	
Reporting	CONSORT statement	STROBE statement	

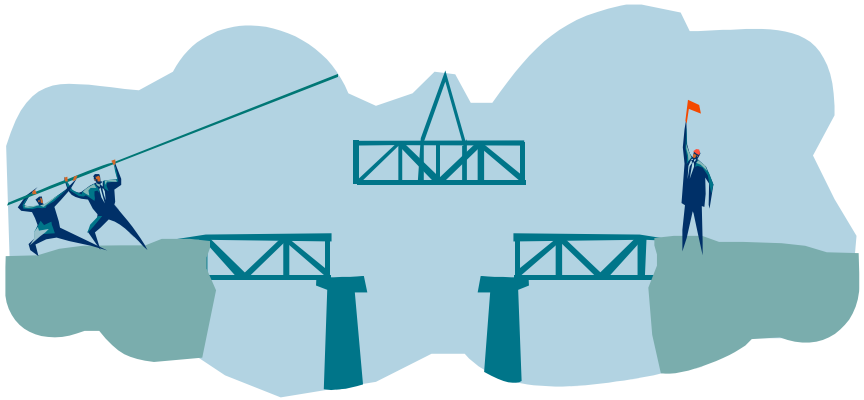
Stakeholder requirements

Regulatory	HTA
Exposure	Burden of target disease (mortality, morbidity prevalence, incidence, DALYs, QALYs)
Epidemiology of the indication(s)	
Prescribing conditions	Conditions of use
Characteristics of patients who actually receive the drug	Expected benefit of the technology - On burden of disease - On management of disease - Economical - Organisational - Social
New safety concerns, known ones, risk factors	
Efficacy in real life / in specific populations	Confirmation of the expected benefit
Effectiveness of risk minimization measures	Potential to cover unmet medical needs or to improve covered needs
Signal detection	

Generating evidence from real world data



Changing the way we are doing studies



Where we are today?

- Budget assigned to uncoordinated outcomes studies to answer specific questions for single products
- Un-coordinated studies conducted across different needs
- Lengthy delays in patient recruitment
- Repetitive or consistently re-worked study protocols
- Separate data sources for each requirement

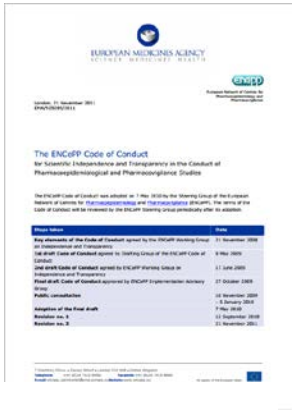
Preparing for tomorrow

- Integrated RWE strategy that builds scalable data assets that are harnessed to generate and test multiple hypotheses
- Accessible data assets to enable rapid response to internal and external stakeholder questions
- Replicable research methods based on highest methodological standards

Connecting stakeholders

European Network of Centres for Pharmacoepidemiology and Pharmacovigilance

Code of Conduct



Good practice

Registration

EU PAS Register

http://www.encepp.eu/encepp/addstudy.jsp

Data sources

ENCePP Resources Database

http://www.encepp.eu/encepp/resourcesDatabase.jsp

Extension into HTA space

ENCePP-HTA working group

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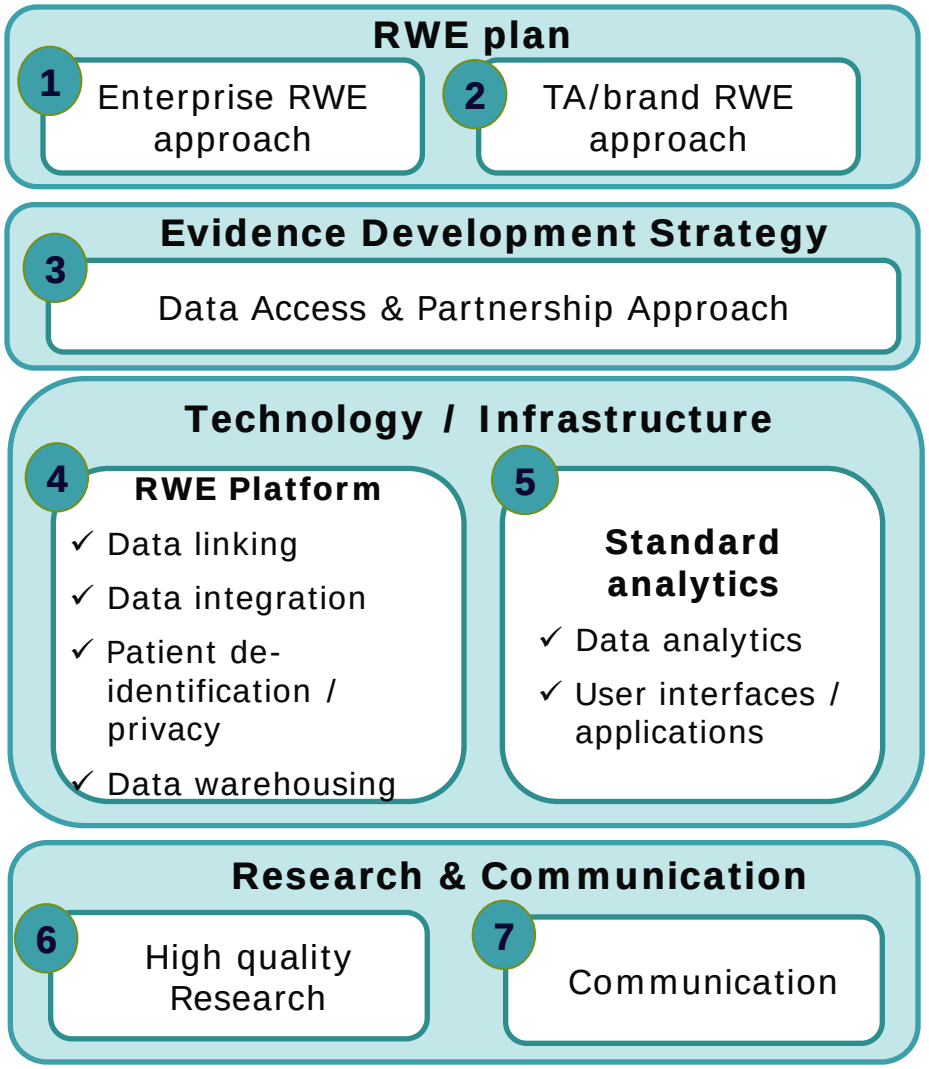
Integrated RWE strategy



1. Define enterprise RWE vision and approach
2. Determine strategic implications and gaps across TAs by including both safety and HTA requirements
3. Develop a strategy around evidence development which includes data access and stakeholder partnering
4. Build platform to securely link, integrate, clean, and house required real world data
5. Utilize data from the RWE initiative across safety, HTA, R&D and commercial needs
6. Develop research based on methodological standards to meet authorities' requirements
7. Use efforts to communicate study results in compliance with codes of conduct

Conclusion

- Real world evidence: a real trend
- Need for harmonization
- Bridging the gap between regulatory and HTA will facilitate
    - Mutual understanding of questions, methods and standards
    - Optimized use of available resources
    - Better assessment of benefit-risk
- Smooth collaboration between stakeholders

Thank you!

Acknowledgments

Thanks to colleagues from ENCePP-HTA working group:
- Yvonne Lis (Parexel)
- Anke Vanengen (Quintiles)

Massoud TOUSSI, MD, PhD, MBA
Medical Director,
Real-World Evidence Solutions / Health Economics & Outcomes Research
IMS Health
Tour Ariane, 5-7 place de la Pyramide, 92088 La Défense Cedex, France
Tel: +33 (0)1-41 35 13 35 • Mobile: +33 (0)6-07 96 66 50 • Fax: +33 (0)1-41 35 13 49
email: mtoussi@fr.imshealth.com

Heterogeneity of choices