

RWE fundamentals. Could the patient be the driving force to drug development?



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Real World Evidence

RWE and importance in drug market

RWE vs Randomized Clinical trials

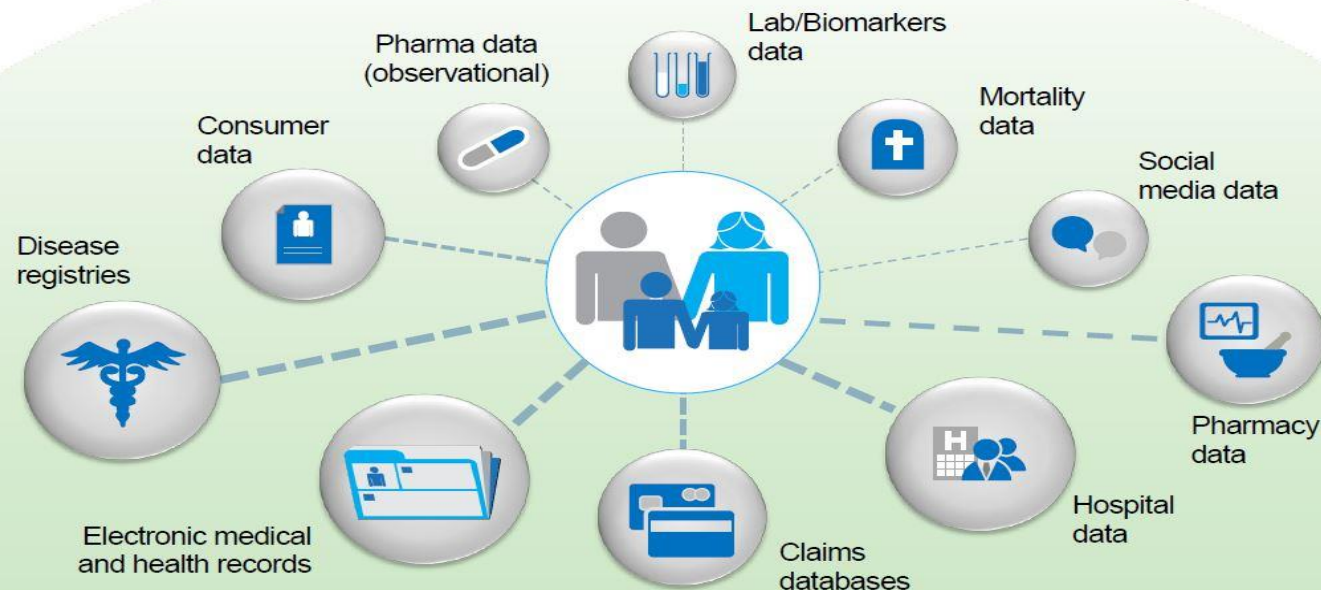
Patient data – drug development

What is RWD?

Real world data is defined as data that are collected outside the constraints of conventional randomized clinical trials.

Sources of Real-World Data (RWD)

RWD is PATIENT data



Source: IMS Health

Who uses RWD?

Clinical Development teams

Healthcare Professionals

Epidemiology and drug safety researchers

Market access

Patient Associations

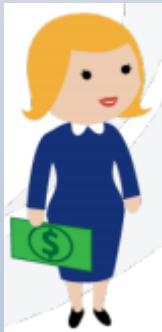
ITS ALL ABOUT THE QUESTION

Does this treatment work?
How safe is it?



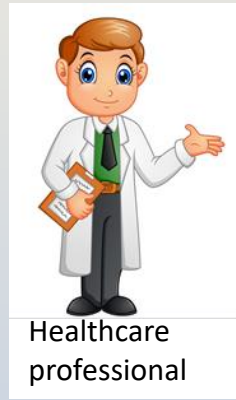
Regulator

How much does this treatment cost? Is it worth it?



Sponsor

How are other physicians using this treatment? What results are they seeing?



Healthcare professional

Is this the best treatment for me?



Patient

Understanding their different perspectives is the key to generating the right evidence

RCTs vs Real World

Clinical Trial



Strict inclusion and exclusion criteria

Strict Study protocol

Strict guidance on difficult cases



Real World



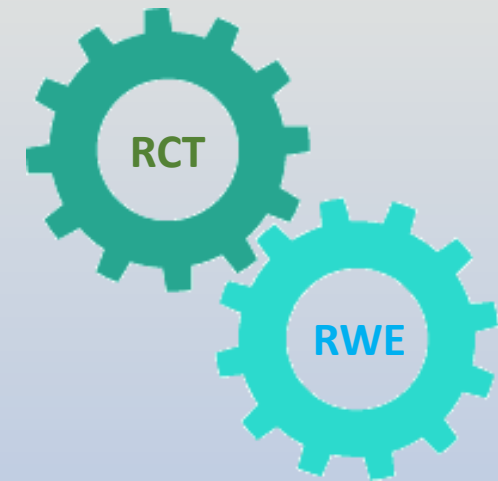
Only indications and contra-indications regulated

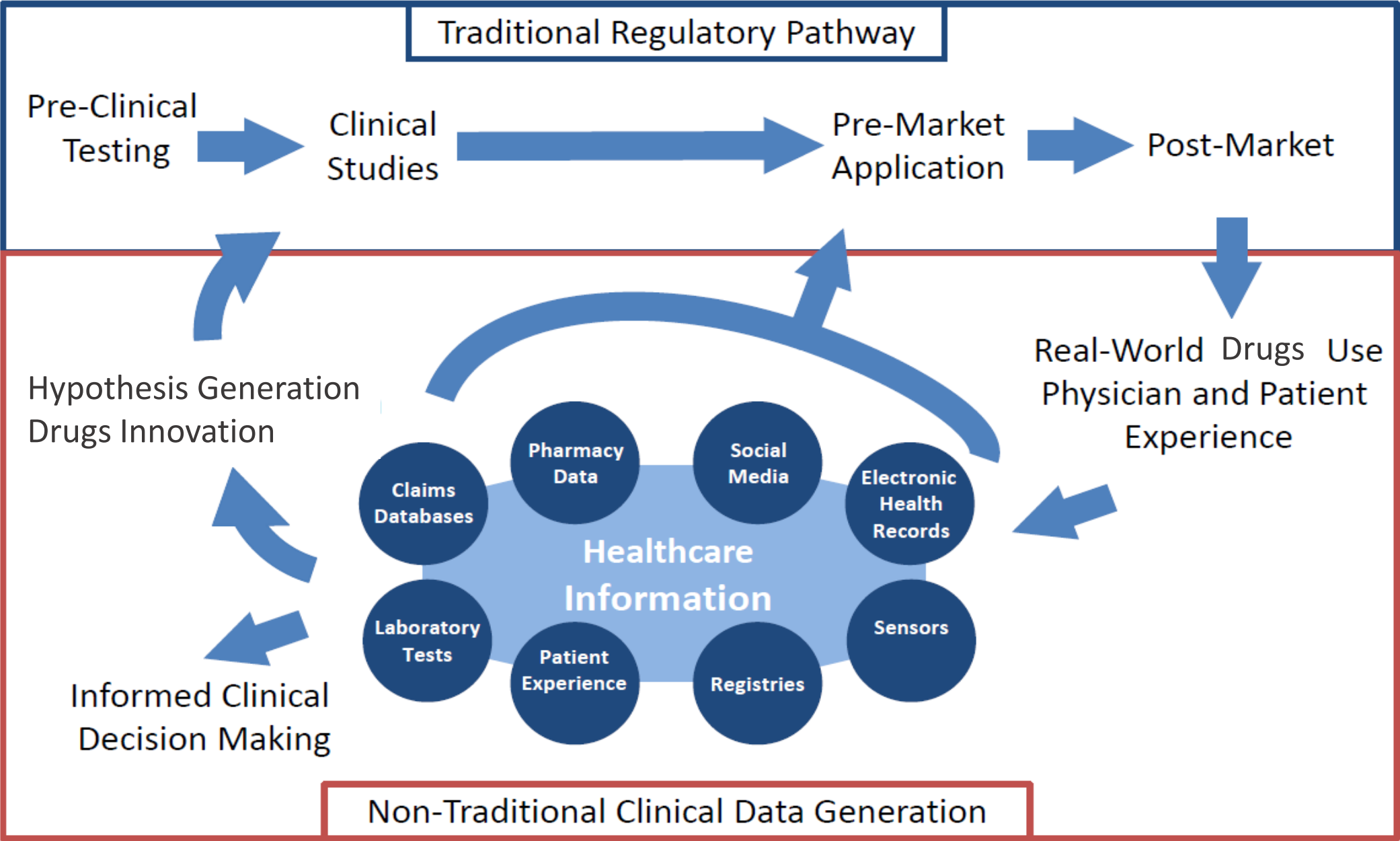
Dose recommendations for groups of patients only

Limited guidance for difficult cases

RWE and RCT data are considered mutually complementary
There are Advantages & Disadvantages

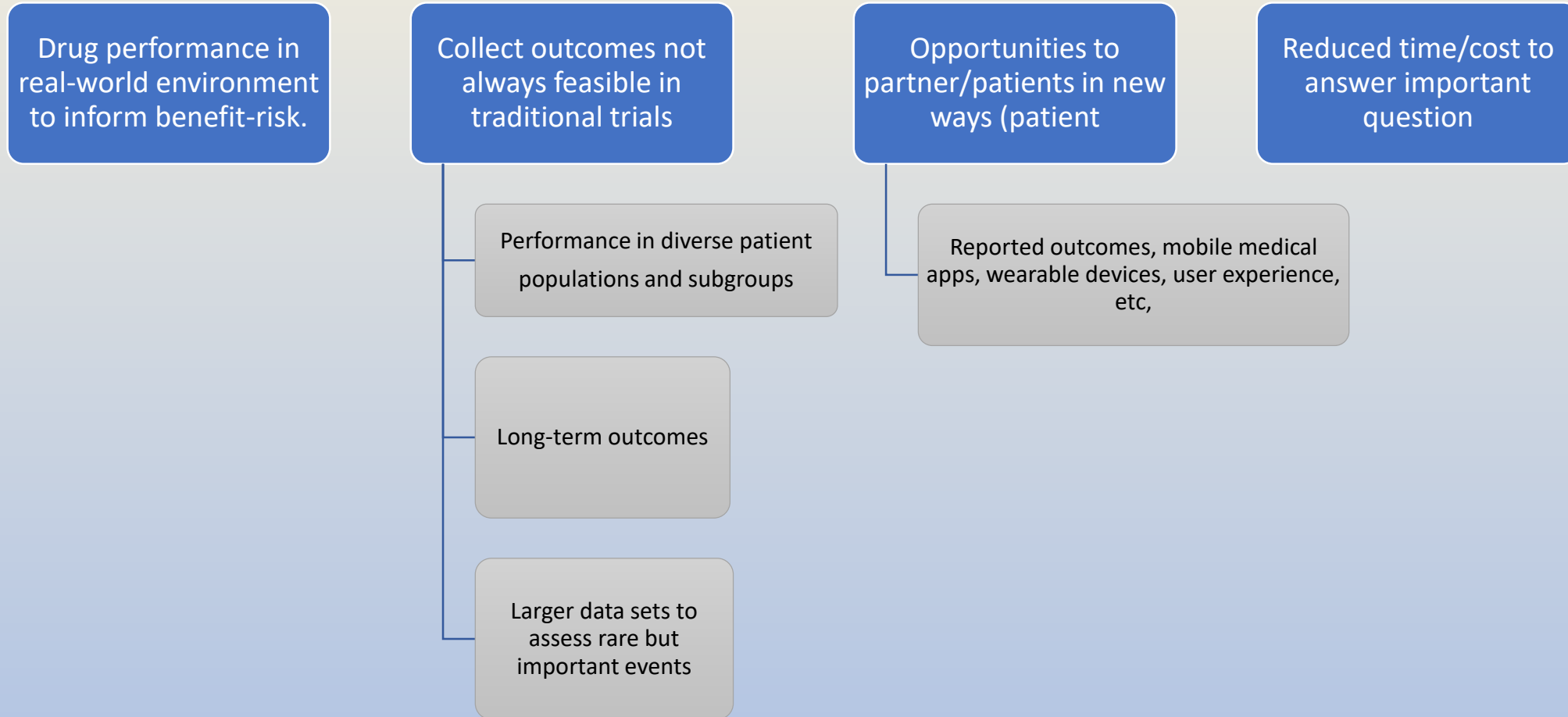
	RCTs	RWE
Objective	Can it work?	Does it work?
Purpose	Regulatory approval	Drug Performance in RW
Setting/design	Ideal conditions (Experimental)	Real world
Intervention	Fixed regimen	Flexible
Compliance	High	Low to High
Study group	homogeneous populations (Restricted)	heterogeneous populations (Wide and unrestricted)
Follow up	Designed	In actual practice
Comparators	Placebo/selective alternative interventions	Many alternative interventions





*FDA RWE Guidance

Potential benefits of RWD sources include:



**FDA RWE Guidance*

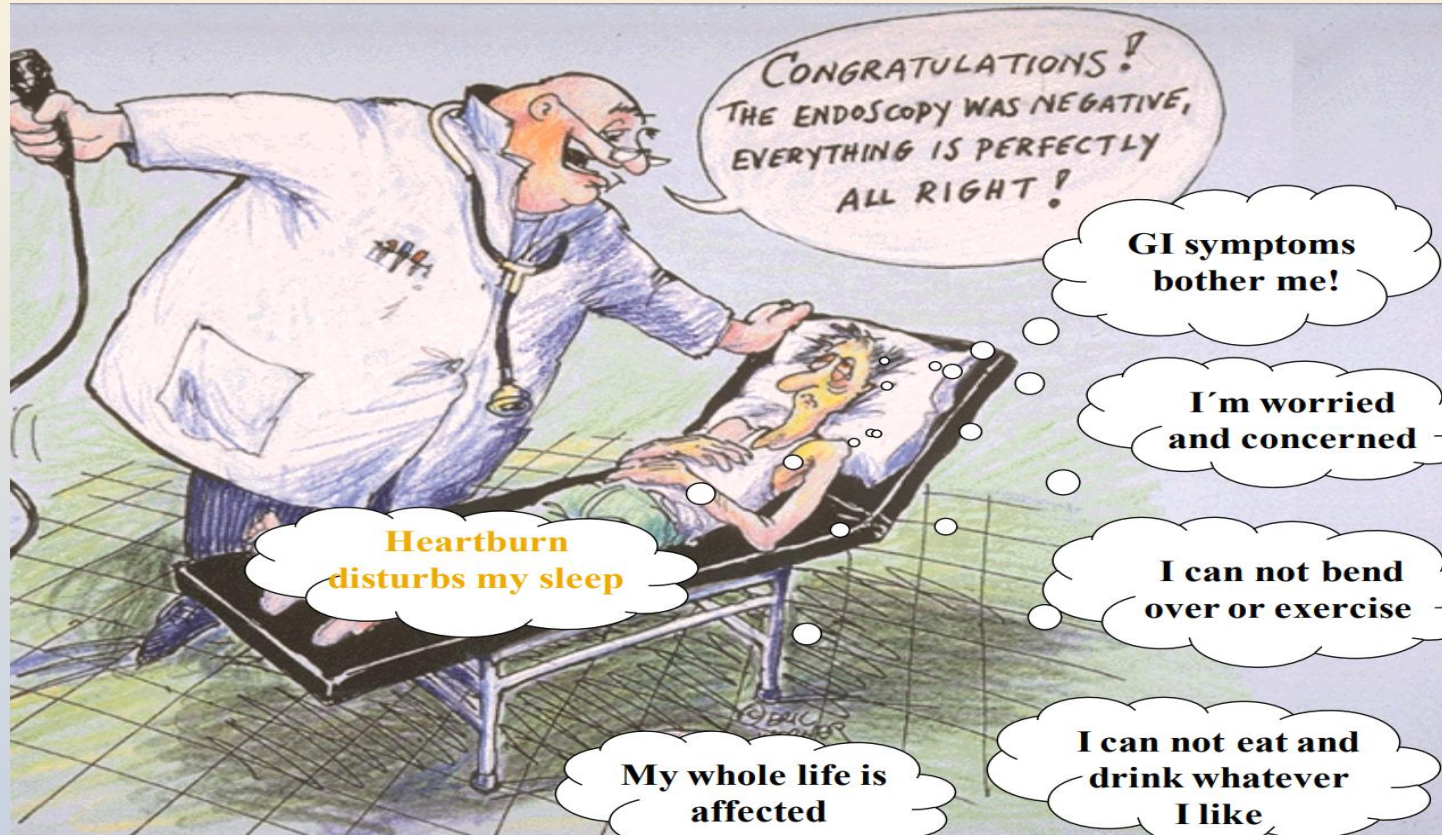
Conclusions

RWE and RCT data are considered mutually complementary

RWE data are important and provide information about medication in the real world

Need to minimize bias (Data Quality Management)

Set the right question



* Hun-Sung Kim, *J Korean Med Sci.* 2018 Aug 20;33(34):e213

* Rachel E. Sherman, M.D., *Real-World Evidence — What Is It and What Can It Tell Us?* *The new England journal of medicine*

* *Oxford PharmaGenesis- The HealthScience Communicators*

* *Surrogate Endpoints in Infectious Diseases Trials: FDA Perspective Surrogate Endpoints in Infectious Diseases Trials: FDA Perspective* John H. Powers, MD.

* <https://www.clinicalleader.com/doc/why-all-the-hype-around-real-world-evidence-here-s-what-you-need-to-know-0001>

* <https://www.pngkit.com/todmca/>

RWE going to an RWE era?
RWE ready?



THANK YOU

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