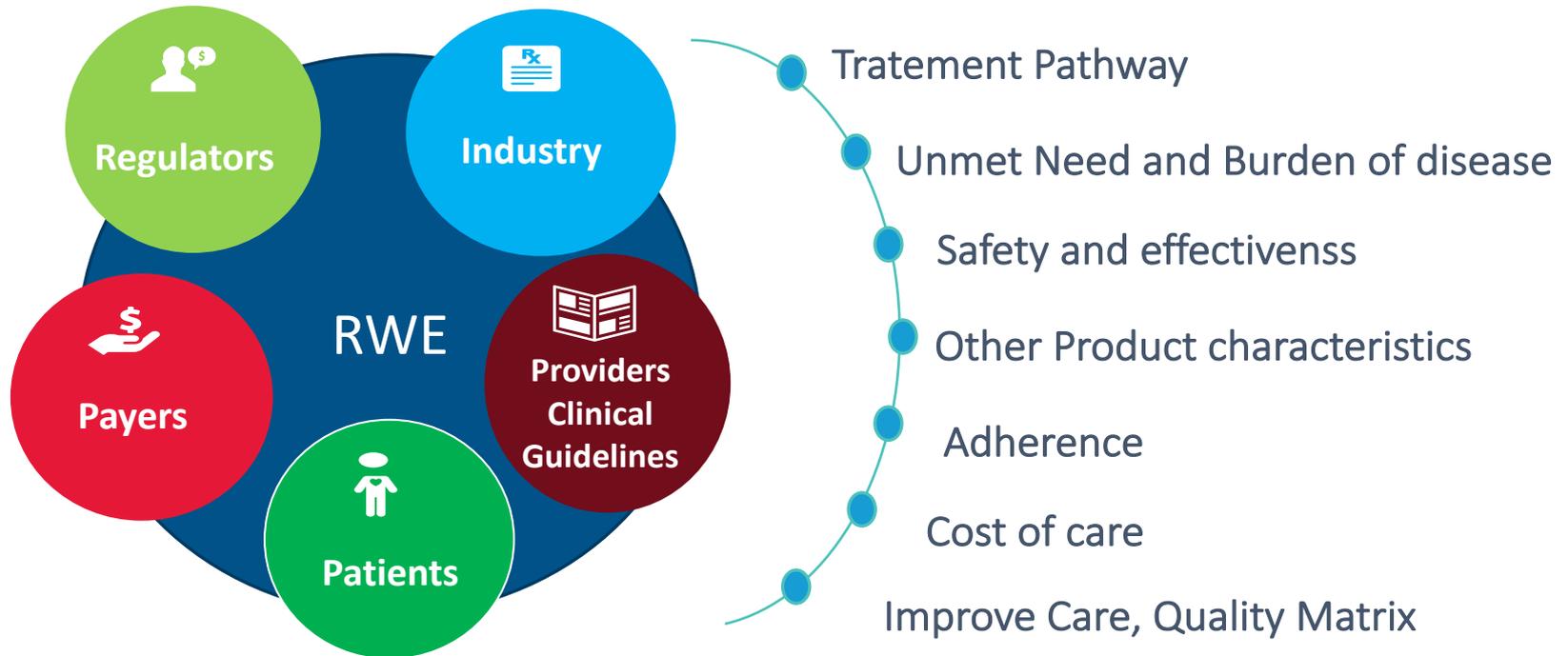


# Experience and Suggestions

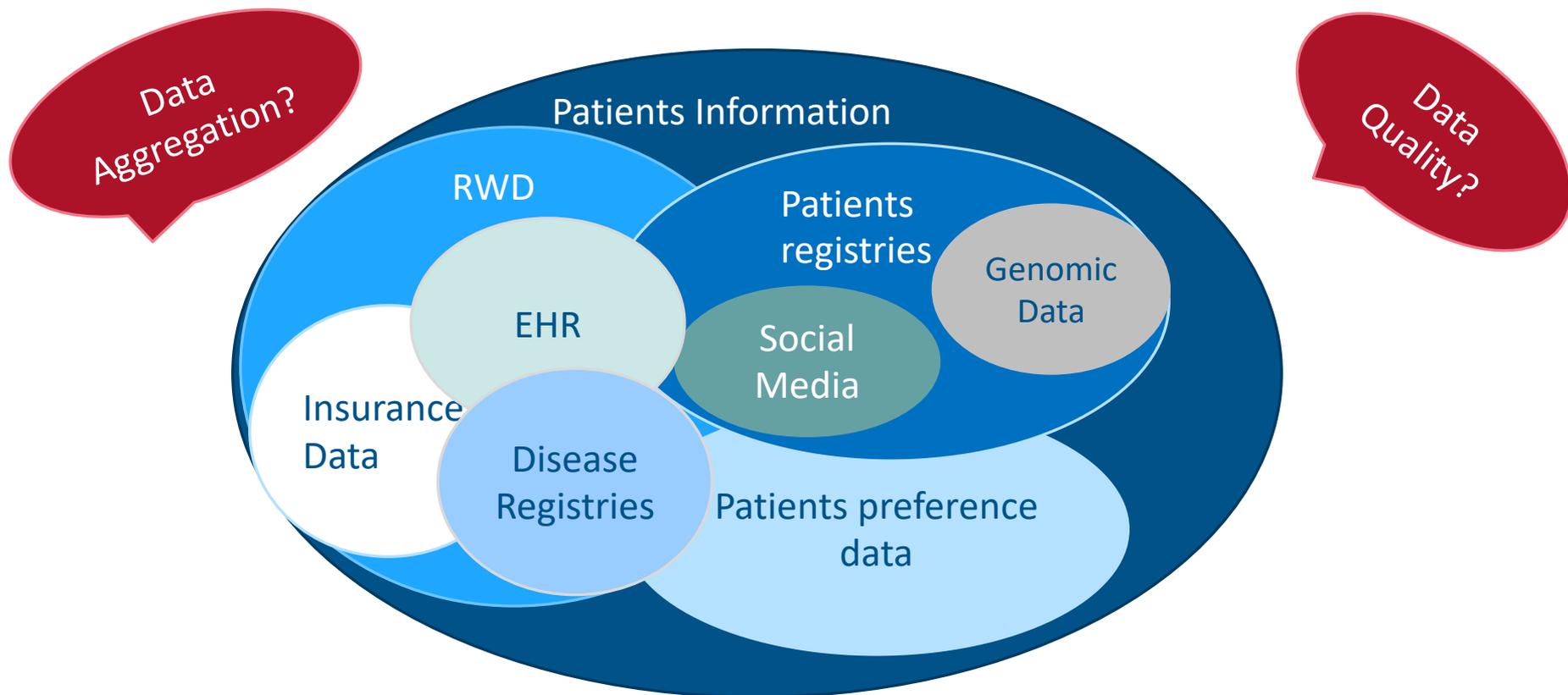
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# Generating RWE across all phases of medicine development



Do we fully understand the disease from patients perspective?

# Complementing/ Supplementing RWD with PPI



# Application of Preference Studies across Drug Development

- Collect systematic data on patients' experiences, preferences, and values
- Ensure study relevance and acceptability to all stakeholders including the patient community
- Support patient-advocacy efforts

Patient engagement and evidence based advocacy

Medical product development

- Clinical trial design
- Product design

Regulatory and reimbursement

- Benefit-risk assessment
- HTA processes

Clinical care

- Treatment guidelines and communication
- Shared-decision making

- Inform endpoint development for use in trial, including meaningful change
- Understand acceptability/ importance of product/treatment
- Predict expected treatment adherence or uptake

- Understand relative importance of treatment side effects and efficacy
- Calculate tolerance for benefit-risk tradeoffs or uncertainty
- Provide data for use in economic evaluation models

- Gain insight into treatment goals important to patients
- Compare patient and physician preferences
- Inform patient-friendly communication strategies and decision aids

Preference studies can be used to integrate patients voice into decision aids and shared-decision making

Source: Jackson et al. (2019) *Expert Review of Pharmacoeconomics & Outcomes Research*

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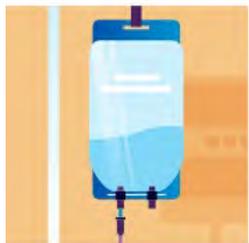
# Patients Preference Studies



What disease aspects are most burdensome to patients and/or have the greatest impact on their quality of life?



How do patients weigh the relative importance of device features?



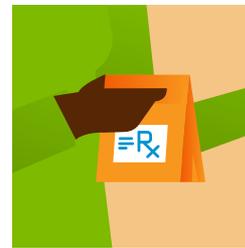
How do treatment preferences vary between earlier and later stages of a disease?



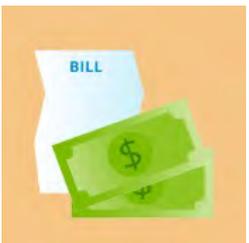
How much risk will patients tolerate for an increase in efficacy and/or convenience? How do they make trade-offs between efficacy and safety?



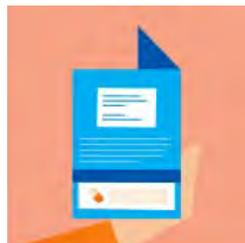
What is the relative importance of efficacy and/or safety endpoints to patients? How do patients allocate weights?



What is the possible patient uptake of a new treatment/device/technology/service?



What is the willingness to pay for an increase in efficacy, a reduction in side effects and/or increased convenience?



How adherent are patients likely to be to a current of future treatment/intervention?

# Using Patients Data Platform to Improve RWE



PATIENTS KNOW BEST®  
MANAGE YOUR HEALTH

Empowering patients to manage their care, enabling professionals to share information while improving efficiencies for payers



## Patients

See your medical records and consult with your team

*Organize your circle of care*

*"Communicating with professionals was a godsend during a period of having hospital tests and getting a diagnosis"*



PKB demo web



# Creating Apps with Patients for Patients

## *Assessment of Real Time Patient Reported Outcomes via a mobile app*

Custom developed App for iPhone and Android platforms with three major components:

**DIARY** - Daily, weekly and monthly patient reported data collection

**COMMUNITY** - Closed community where participants can share their experiences and message each other. Designed to emulate familiar social platforms

**MATERIALS** - Resource for participants as it relates to our drug and the study including patient videos, drug information, tutorials, and FAQs

*Madeline*  
Mobile App Diary of Everyday Life  
and Experiences

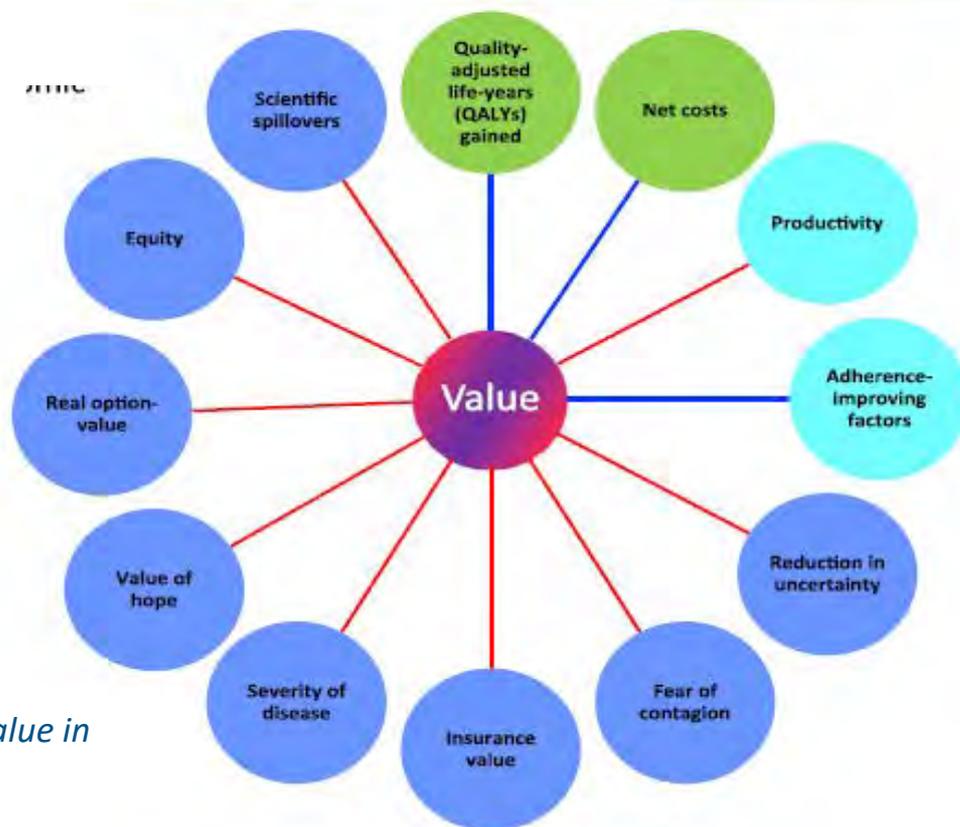


### Key Features

- ☑ Simple set up, easy to use for elderly patients
- ☑ Engaging interface - reduces recall bias and boredom associated with long paper questionnaires
- ☑ Provides real time trends on mood, fatigue, and pain back to patients
- ☑ Has ability to connect with activity trackers (e.g. Fitbit, JawBone)
- ☑ Scalable for wider use outside current study

# PPI can improve RWE by enhancing the opportunity to assess the elements of value

## Elements of Value



Source: Garrison et al. (2018) *Value in Health 21*