Experience and Suggestions
Generating RWE across all phases of medicine development

Regulators  Industry  Payers  Providers Clinical Guidelines  Patients

- Treatment Pathway
- Unmet Need and Burden of disease
- Safety and effectiveness
- Other Product characteristics
- Adherence
- Cost of care
- Improve Care, Quality Matrix

Do we fully understand the disease from patients perspective?

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Complementing/Supplementing RWD with PPI

- Patients Information
- Patients registries
- Social Media
- Genomic Data
- Data Aggregation?
- Data Quality?
Application of Preference Studies across Drug Development

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

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

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

How do patients weigh the relative importance of device features?

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

How adherent are patients likely to be to a current of future treatment/intervention?
Using Patients Data Platform to Improve RWE

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"
Creating Apps with Patients for Patients

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

**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

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