Helping Payers use Real-World Data to Make Formulary Decisions

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BACKGROUND

Pharmacists at health insurance companies review research studies and other information to make decisions about which medications offer the best effectiveness for the cost and how to pay for them. They are interested in real-world data from patient care because it shows what happens in normal patient treatment that might be different from clinical trials.

OBJECTIVE

We gathered a group of experts from pharmaceutical manufacturers and health insurers to participate in discussions. The goal was to learn how to help them use real-world data, and what kind of information is most useful for health insurers to include in medication coverage decisions.

KEY POINTS

- **Real-world data** comes from patient charts and other sources, like questionnaires, used to document patient care at regular doctor or hospital visits.
- Scientists use real-world data that can no longer identify individuals but can be used to understand diagnosis, treatments, and test results during regular patient care visits.
- Real-world data can provide different information than information from clinical trials and can help insurance payers and doctors make better decisions on the most appropriate treatment for their patients.

PATIENT/COMMUNITY IMPACT

- It is important to know that decision-makers at health insurance companies are interested in using real-world data
- Clinical trials are very important and necessary, but real-world data adds information not always available from a clinical trial.
- The information from real-world data provides a more complete picture of the patient's experience when they are treated for their disease(s).
- Patients should feel comfortable with scientists using real-world data collected from regular visits to their doctor. All information that could identify a specific patient is removed before the scientists use real-world data.

PROJECT DESIGN

Focus Group #1

A standard set of instructions are needed:
- Describing what real-world information is most useful for pharmacists making decisions on what drugs to include in health plan coverage
- Information on how to analyze real-world evidence
- Suggestions for how pharmaceutical companies should communicate real-world evidence to payer pharmacists

Focus Group #2

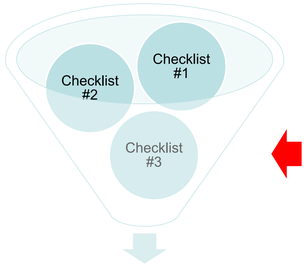
Specific information to include:
- A list of examples of real-world study types available for formulary decisions
- A list of different information that could be available at different points during drug development and after FDA approval
- A checklist and worksheet including information that pharmacists at health plans find most useful

Workshop

Participants tested the standards to see how they could be used:
- Describing the process for scientists from pharmaceutical companies to fill out the checklist
- Describing how payer pharmacists can use the information as part of their formulary decisions

PROJECT HIGHLIGHTS

AMCP RWE Standards = Framework + Criteria

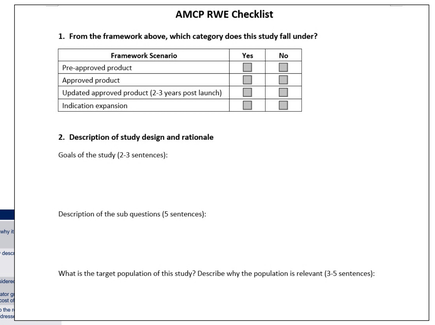


29 Final Criteria

We reviewed other publications that had guidelines or checklists related to real-world data:
- None were specific for payer decision-making
- We chose criteria that were most relevant
- We refined the criteria through consensus among all (about 50) people participating in the focus groups and workshop

We made the criteria into a checklist so it would be easier to use

Questions include:
- What are the limitations of your study?
- What data source(s) was used? Why was it relevant/appropriate for the research question?



AMCP RWE Checklist

1. **From the framework above, which category does this study fall under?**

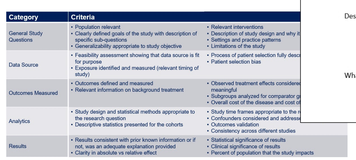
Framework Scenario	Yes	No
Pre-approved product	☐	☐
Approved product	☐	☐
Updated approved product (2-3 years post launch)	☐	☐
Indication expansion	☐	☐

2. **Description of study design and rationale**

Goals of the study (2-3 sentences):

Description of the sub questions (5 sentences):

What is the target population of this study? Describe why the population is relevant (3-5 sentences):



Category	Criteria	
General Study Questions	• Population relevant • Clearly defined goals of the study with description of specific sub-questions • Generalizability appropriate to study objective	• Relevant interventions • Description of study design and why it • Settings and practice patterns • Limitations of the study
Data Source	• Feasibility assessment showing that data source is fit for purpose • Exposure identified and measured (relevant timing of study)	• Process of patient selection fully descr • Patient selection bias
Outcomes Measured	• Outcomes defined and measured • Relevant information on background treatment	• Observed treatment effects considere meaningful • Subgroups analyzed for comparator g • Overall cost of the disease and cost o
Analytics	• Study design and statistical methods appropriate to the research question • Descriptive statistics presented for the cohorts	• Study time frames appropriate to the r • Confounders considered and address • Outcomes validation • Consistency across different studies
Results	• Results consistent with prior known information or if not, was an adequate explanation provided • Clarity in absolute vs relative effect	• Statistical significance of results • Clinical significance of results • Percent of population that the study impacts

Final Framework

We created a framework to list the main study types and endpoints most useful throughout drug development and real-world use:
- Pre-approval = before a drug is FDA approved
- Launch = after a drug is FDA approved and available for use
- Post-approval = After a drug has been in real-world use for a period of time
- New indication = An approved product is investigated for use in a new disease or treatment

	Pre-approved Product Dossier	Approved Product Dossier	Updated Approved Product Dossier	Indication Expansion Dossier
General Timeline	1+ years prior to launch	At product launch	2+ years post launch	1+ years post launch
Study Type (Examples)	• Epidemiology and natural history of disease, e.g., real-world diagnosis and treatment patterns • Description of clinical outcomes associated with standard of care (SoC) • Subgroup analyses • Real-world patient experience • Total cost of care analyses • Economic models	• Pragmatic clinical trial • Adaptive trials • External control arm • Indirect treatment comparison • Analyses of outcomes of patients treated under compassionate use programs • Subgroup analyses • Biomarker validation studies • Real-world patient experience • Economic models	• Real-world safety and effectiveness studies (single arm or comparative) • Roll-over studies/long-term extension • Subgroup analyses • Real-world endpoint validation studies • Real-world patient experience • Meta analyses of real-world evidence or clinical trials • Economic models	Current standard of care • Same as pre-approved product dossier From off-label use / RW data • Real-world safety and effectiveness (single arm or comparative studies) • External comparator • Indirect treatment comparison • Subgroup analyses • Biomarkers • Real-world patient experience
Endpoints	Disease epidemiology • Current SoC • Comorbidities • Unmet need • Burden of illness • Drug utilization/treatment patterns • Adherence to treatment (e.g., discontinuation, switching) • Major safety events Economics • HCRU • Cost-effectiveness/budget impact of disease or SoC • Total cost of care	• Endpoints from pre-approval dossier with direct or indirect comparison to clinical trial outcomes	Clinical • Refined description of treated population • Adherence and persistence • Validation of surrogate endpoints that may have been included in product approval • Real-world effectiveness • Real-world safety Economics • HCRU • Cost-effectiveness/budget impact of disease or SoC • Total cost of care	Clinical • Refined description of treated population • Adherence and persistence • Validation of surrogate endpoints that may have been included in product approval • Real-world effectiveness • Real-world safety Economics • HCRU • Cost-effectiveness/budget impact of disease or SoC • Total cost of care

CONCLUSIONS

- Real-world evidence can provide information on patient experiences in normal clinical care
- These standards will help pharmaceutical companies design studies that generate information that is most useful for decision-making
- These standards will help pharmacists at health plans use real-world evidence as part of their formulary decisions

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