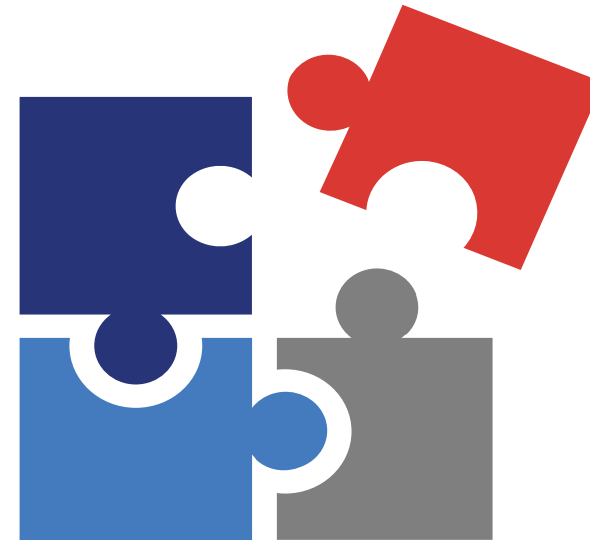


STUDIES INCORPORATING THE PATIENT VOICE PROVIDE VALUE

To Patients

To
Physicians



To Medical
Product
Development
Companies

To
Regulatory/
Payers



Making the Patient Voice Count

DEMONSTRATING WHEN, WHY, AND HOW TO ENGAGE PATIENTS

Presenters:



Caitlyn Solem, PhD
Principal & Executive Director
Patient-Centered Outcomes



Kelly McCarrier, PhD, MPH
Director & Qualitative Research Lead
Patient-Centered Outcomes

Now who are you?



Patient-Centered Outcomes Studies are Integral to HEOR Value Plan



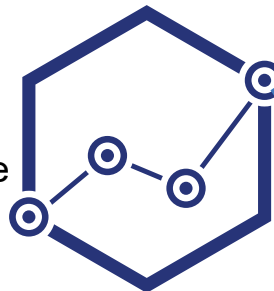
Strategic Market Access

- Patient journey provides context in dossiers
- Patient perspective / participation in drug development and approval process increasingly valued and required by regulatory bodies worldwide



Real-World Evidence

- Incorporate custom or validated instruments to understand real-world outcomes
- Qualitative research to characterize patient experience



Economic Modeling

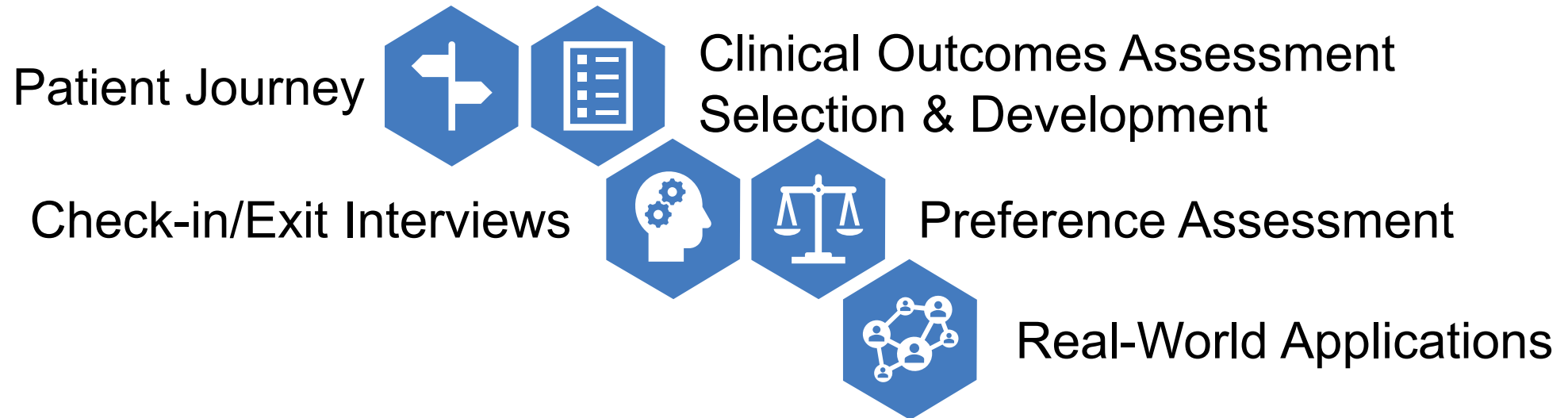
- Patient journeys can help to inform core health states
- Preference research leading to utilities for key health states



Our Goal is to Support Implementation



Study Types



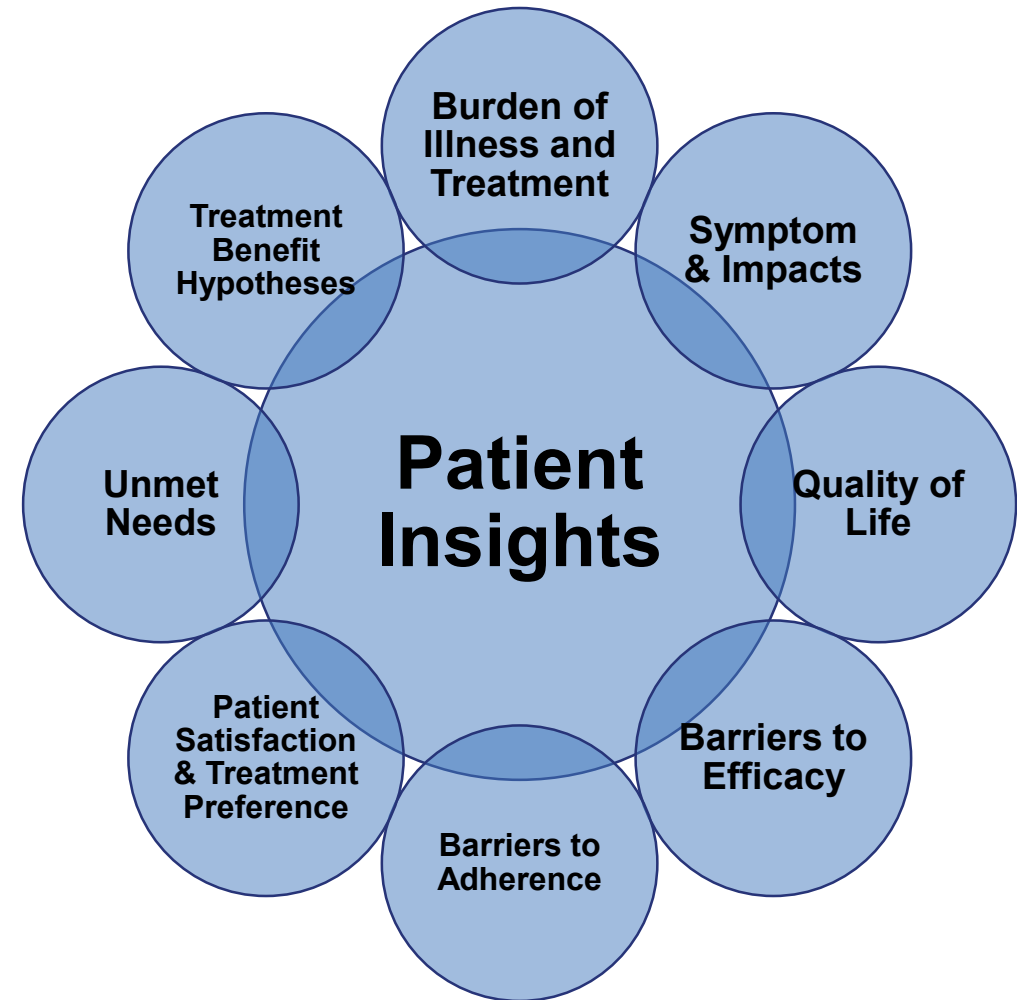


Patient Journey Studies



Insights from Patient Journey Studies Can be Varied

- **What:** Exploratory qualitative interview
- Hypothesis generating
 - Foundational to other types of data collection effort





Use Clinical Outcome Journey Studies to Obtain Insights into Patient Experience

► Value:

**Clinical
trial
design**

- Endpoint measurement
- Safety / AE monitoring strategy
- Study visit schedules

**HEOR
strategy**

- Identify research gaps
- Focus research efforts on patient needs

**Treatment
benefit
story**

- Characterize burden of illness
- Inform qualitatively treatment benefit hypotheses





Clinical Outcomes Assessment (COA) Selection and Development

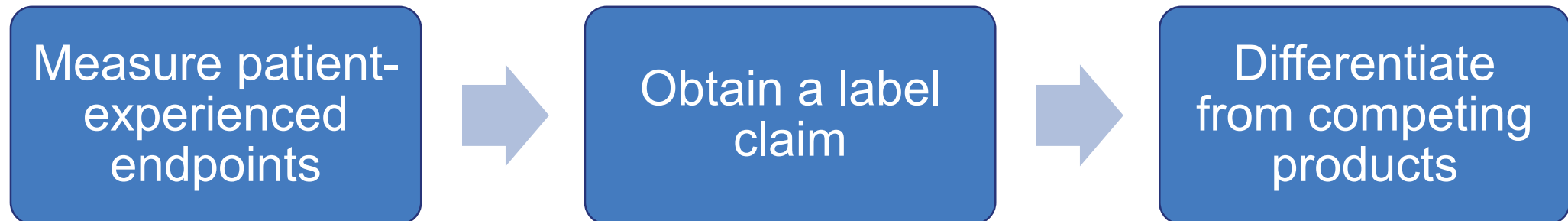


Selection of Appropriate Clinical Outcomes Assessment Drives Potential Product Differentiation

► What:

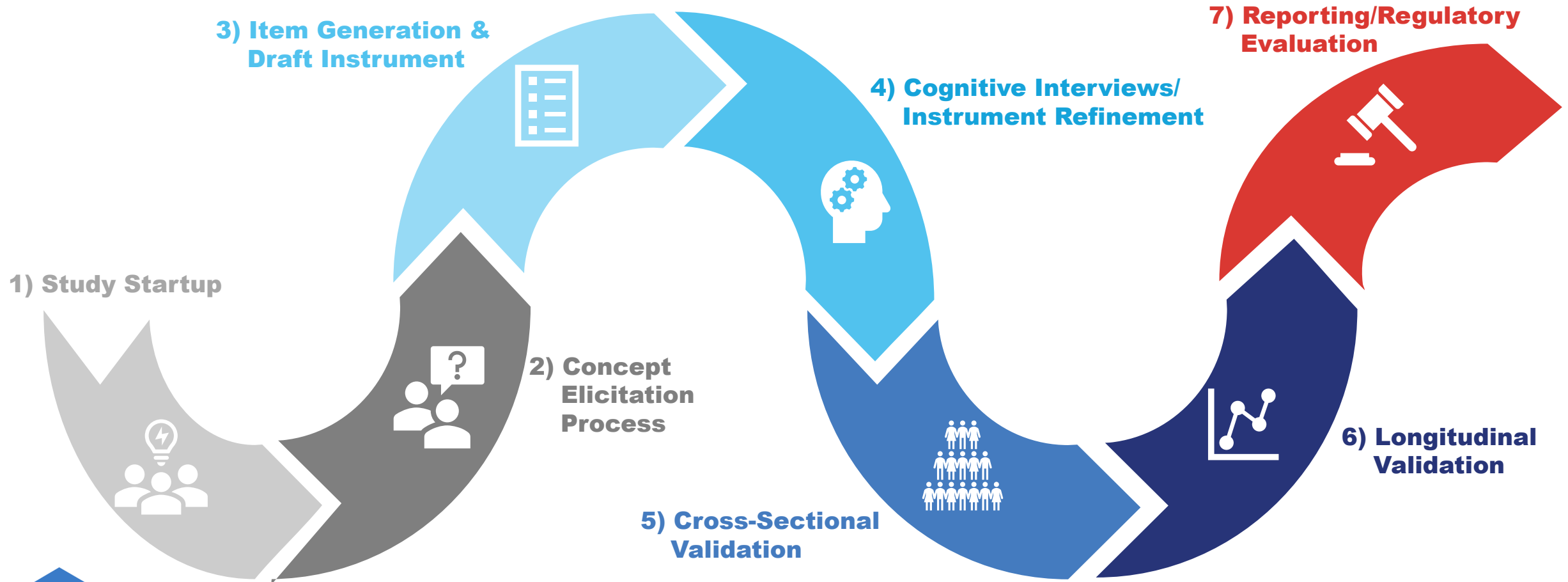
- Review of available COAs (patient, clinician, or observer-reported)
- Develop new instrument to measure patient symptoms or impact of treatment (as warranted)
- Guidance available on how to appropriately conduct
 - PRO Guidance
 - COA Qualification Guidance
 - ISPOR Content Validity Task Force

► Value:





Specific Steps Required to Develop, Refine, and Evaluate Instrument with Patient Input



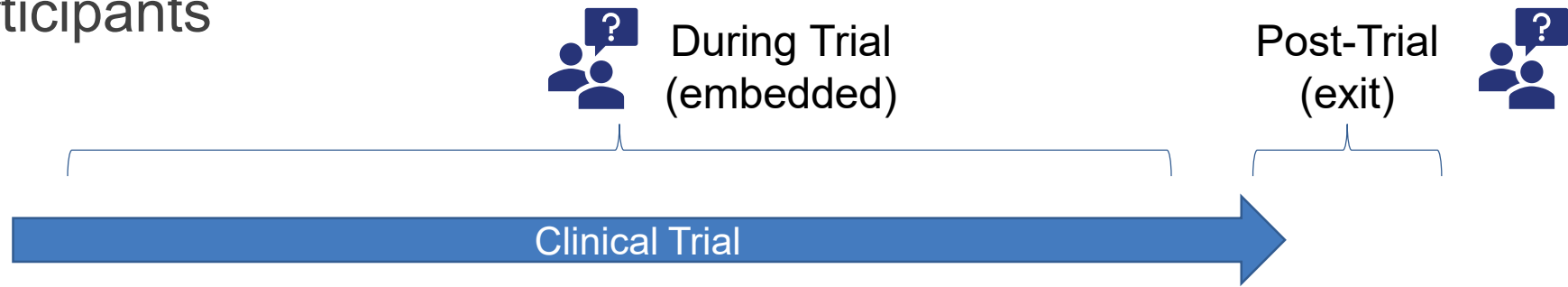


Trial Participant Interviews

Trial Participant Interviews Offer Unique Insight



- ▶ **What:** Qualitative interviews conducted with current or recent clinical trial participants



- ▶ **Value:**

Experience with trial process

Barriers to study adherence / retention

Sources of missing data / measurement error

Experience with treatment effect

Unexpected treatment benefits / side effects

Expectations, preferences, satisfaction

Meaningful change from patient perspective





Preference Assessment



Preference Assessment Methods Quantify Patient Decision Making Processes

- ▶ What: Quantitative methodologies to measure the preferences a patient has when comparing
 - Health states
 - Treatment attributes

▶ Value:

Elicit utilities for health states

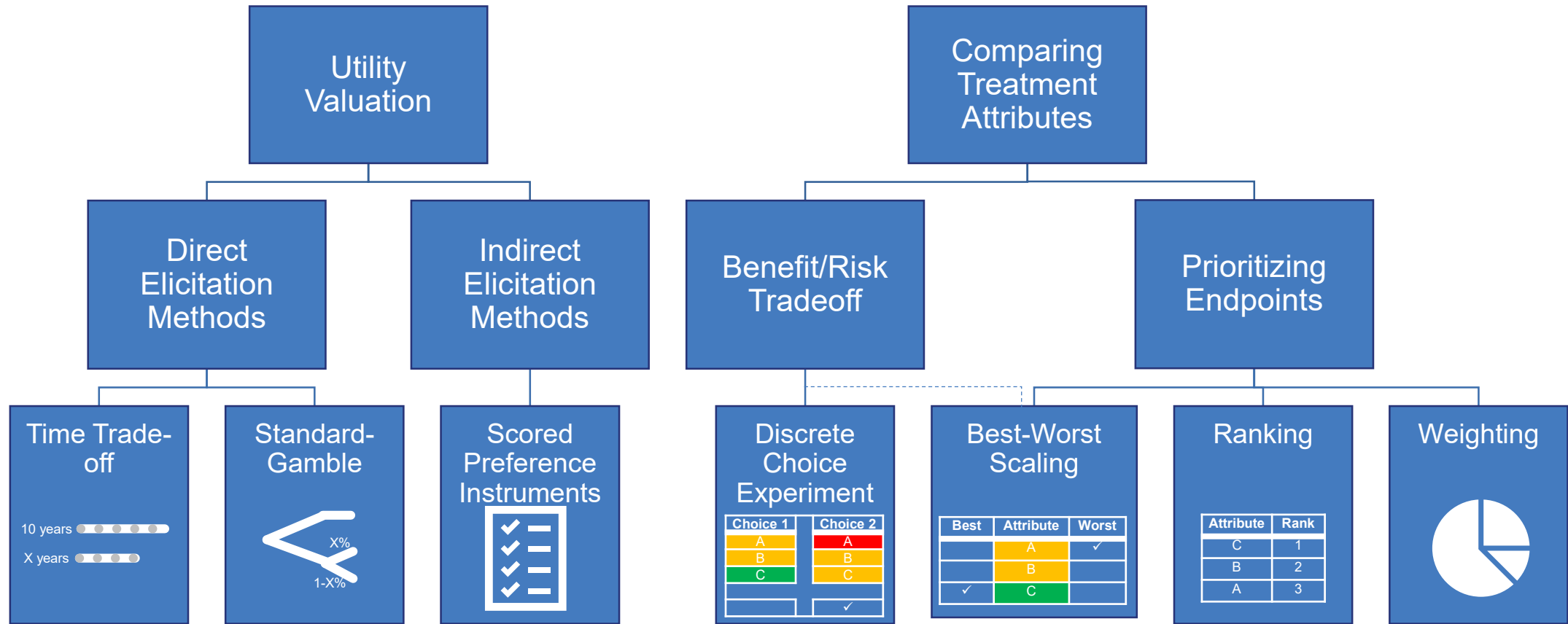
Inform benefit-risk assessment

Understand patient priorities when making treatment decisions





Many Paths Exist to Elicit Preferences



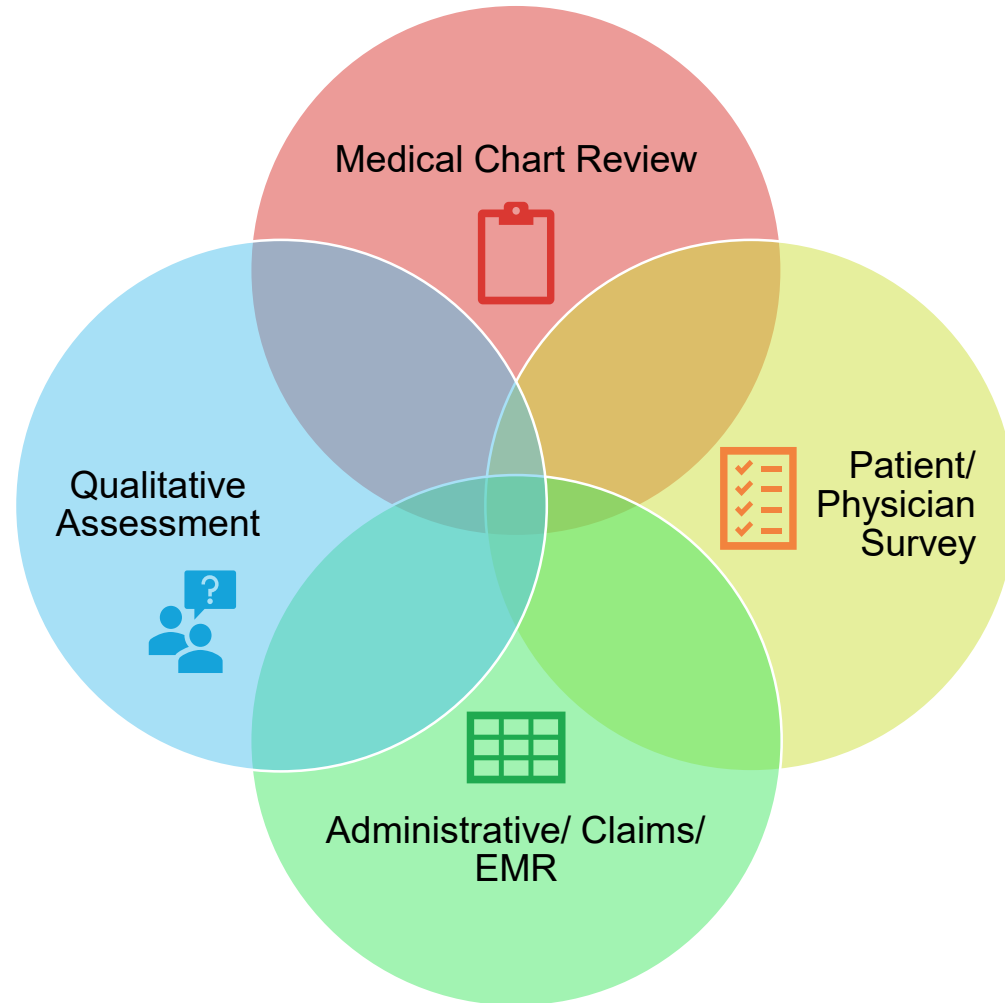


Real-World Data Integrations



Real-World Data Integration

WHAT



▶ Value

- Demonstrate burden of illness
- Provide historical control data
- Contextualize clinical and humanistic endpoints

▶ Key Considerations

- Data linkages/ impact on generalizability
- Cross-sectional vs. longitudinal
- Countries
- Rarity of condition





In Conclusion

Important to Plan Early



Patient Journey
4-6 Months



Instrument Development
24-36 Months



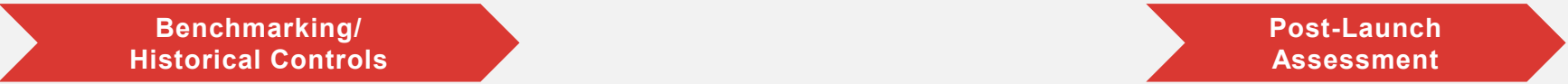
Embedded/Exit Interviews
6+ Months



Preference Assessment
10 -12 Months



Real-World Data Integration
12 - 36 Months



Incorporating Patient Input Elevates the Quality of Decision Making

If you need to...

Characterize burden of illness

Inform treatment benefit hypotheses

Identify unmet needs

Measure patient-experienced endpoints

Differentiate from competing products

Obtain a label claim

Gain first-hand perspective on treatment benefit

Mitigate missing data or measurement error

Understand meaning of change on endpoints

Elicit utilities for health states

Inform benefit-risk assessment

Understand importance of different attributes

Provide historical control data

Connect clinical and economic to humanistic outcomes

Demonstrate burden of illness

Consider...



Patient Journey Studies



COA Instrument Development



Trial Participant Interviews



Preference Assessment



Real-World Patient Data Integration



Thank you! Questions?



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