

Eliciting Value Elements for a Patient-Driven Value Assessment

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PATIENT-DRIVEN
VALUES *in* HEALTHCARE
EVALUATION

Patient-Driven Values in Healthcare Evaluation (PAVE) is a Center of Excellence in Patient-Driven Value Assessment funded by the Pharmaceutical Research and Manufacturers of America (PhRMA) Foundation

- Value frameworks have been developed to aid health care stakeholders in assessing the value of new treatments
- Existing value frameworks have been criticized for not adequately incorporating the patient perspective
 - Outcomes patients care about are not included¹
 - Patients are not contributing to the development of the framework *from conception*²
 - Patients are not engaged *throughout* the creation of the framework²

1. Perfetto EM, et al. Value to Whom? The Patient Voice in the Value Discussion. Value Health. 2017;20(2):286-291.

2. National Health Council. The patient voice in value: the National Health Council patient-centered value model rubric. March 2016.

- To elicit the value elements in healthcare decision-making that are important to the patient community
- To prioritize the patient-driven value elements
- To refine the phrasing and definitions of the patient-driven value elements



Approach:

- Value elements were elicited and prioritized in 3 phases:
 - **Phase 1:** *Develop a List of Existing Value Elements from the Literature*
 - **Phase 2:** *Elicit Elements of Value from Patient Stakeholder Advisors*
 - **Phase 3:** *Prioritize and Refine the Value Elements*

Stakeholders:

- Patient Stakeholder Advisory Committee Members (3)
 - One member represented the Hispanic community
 - Two members were caregiver advocates
- National Health Council (NHC) Value Workgroup Members (14 diverse patient communities)

Develop a List of Existing Value Elements from the Literature



**Phase
1**

- **A review of value elements reported in the literature provided a foundation from which to compare with the patient-informed value elements**
- Value elements used in existing value frameworks and economic evaluations that have incorporated patient-driven or patient-centered value elements were reviewed

Elicit Elements of Value from Patient Stakeholder Advisors



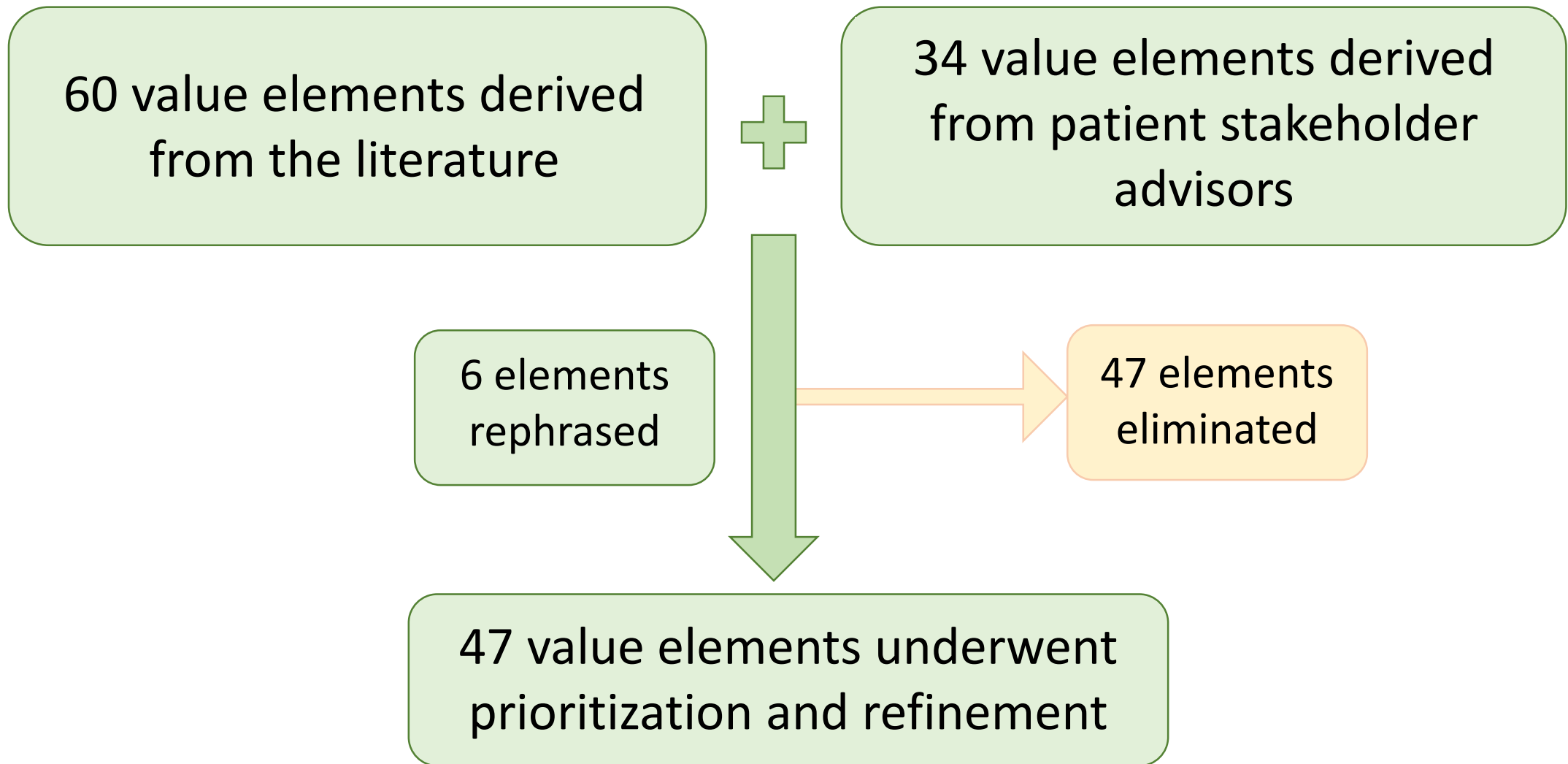
- We engaged our 3 Patient Stakeholder Advisory Committee Members
- The patient stakeholders provided input *via* an iterative process:
 1. Patient stakeholders provided initial input on the value elements important to patients, relevance of each literature-derived value element, and definitions for all value elements
 2. The list of value elements was revised
 3. Patient stakeholders provided input on the revised list
 4. Patient stakeholders' comments were consolidated and the list was revised again

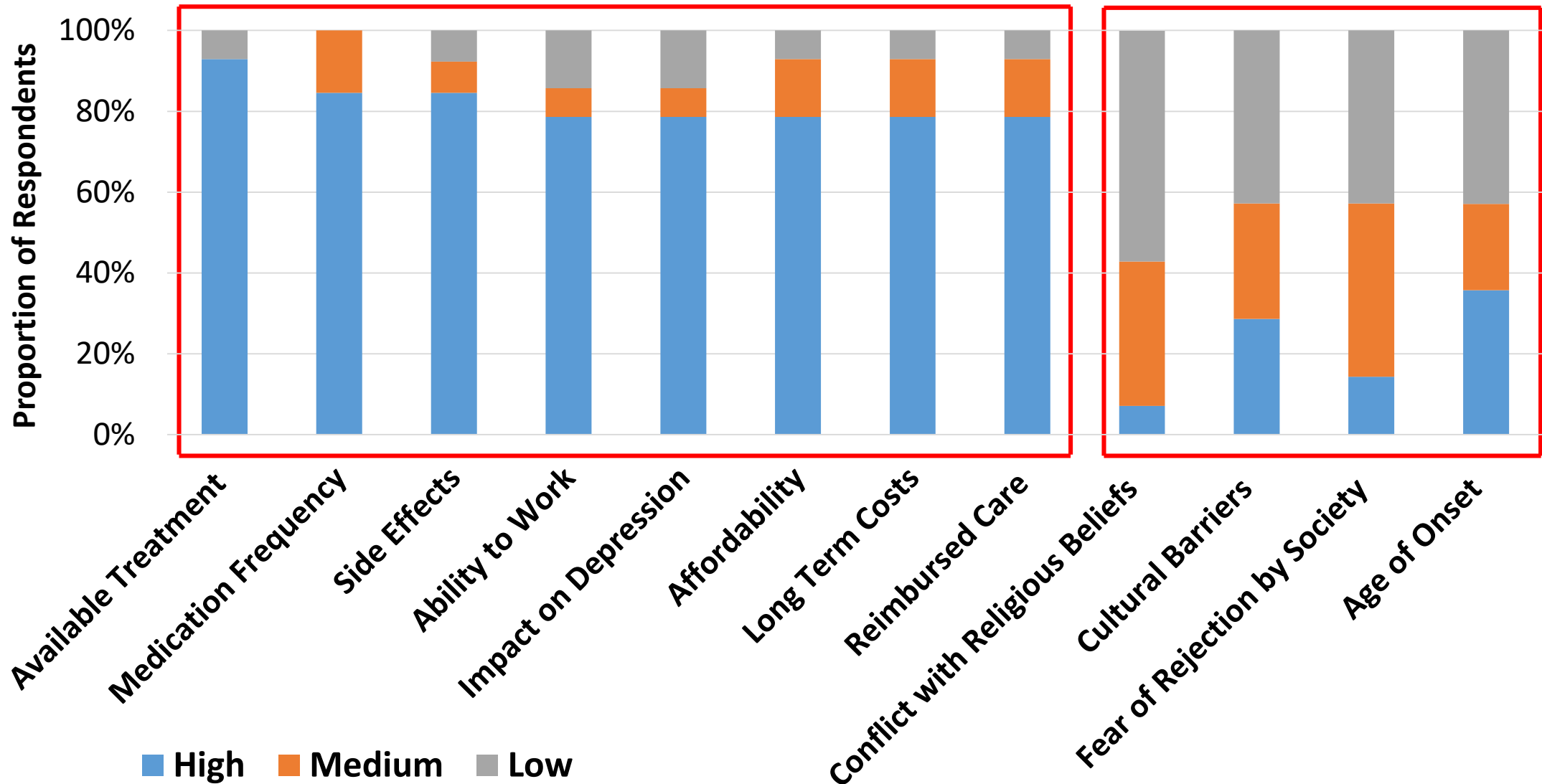


Prioritize and Refine the Value Elements



- NHC Value Workgroup Members representing diverse patient communities were provided a list of value elements and their definitions *via* an online tool (Qualtrics), and asked:
 - How important to treatment decision-making is this value element to the patient community? (High/Medium/Low)
 - Would you rephrase the element label or the definition? (Yes/No)
 - If yes, how would you rephrase the element label or the definition?
- Responses were obtained between September 2018 – November 2018







"Would you rephrase the element label or the definition?"

Value Element	Definition	Yes N(%)
Intermediate/ Surrogate Endpoints	A treatment endpoint that may correlate with a true endpoint, but does not always guarantee the true endpoint will be achieved.	8 (66.7%)

- Examples of feedback received from the patient community:
 - “A treatment endpoint that may correlate with a true endpoint and **while reasonably likely to predict a clinical benefit, it may not guarantee the true endpoint will be achieved due to uncertainty**”
 - Clarify what is meant by “**endpoint**”
 - “**Type of measurement**” instead of “endpoint”
 - “**Decision point**” instead of “endpoint”



"Would you rephrase the element label or the definition?"

Value Element	Definition	Yes N(%)
Side effects	The burden that the effects of medication present.	7 (53.9%)*

*1 missing response

- Examples of feedback received from the patient community:
 - **“Negative reaction** that may accompany treatment”
 - **“Unintended effects** of a medication”
 - **“Feel”** instead of “burden”

- A patient-informed list of value elements was derived from an iterative qualitative assessment with diverse patient communities
- Patient-driven value elements that were of highest importance to patient communities included the tolerability of the treatment, functional impact on personal well-being as well as cost and access to available treatments
- Next steps:
 - Elicit the value element priorities for specific disease conditions and assess the benefit-risk tradeoffs among these elements
 - Incorporate patient-driven value elements into existing value frameworks for a more patient-driven evaluation of treatment effectiveness
 - Test novel methodological approaches for including patient-driven value elements into economic evaluations