

Responding to Stakeholder Input: Finding the Patient Voice in ICER's Value Assessments

September 2018



Background & Methodology

Despite being vital to achieving patient-centered health care, patient engagement in value framework evaluations has often been limited.

- Value framework developers typically utilize traditional analytic approaches to measure the value of treatments and services in an effort to aid stakeholders seeking to standardize health care decisions (commonly at the U.S. payer level).
- Although labeled as ‘value assessment frameworks,’ the Institute for Clinical and Economic Review (ICER), NCCN, DrugAbacus, and others may not fully capture important aspects of care that patients value.
- In fact, several have been criticized in the past for failing to align with stakeholder efforts to move toward a more patient-centric health care system and advance access to personalized and precision medicine.
- PIPC previously set forth recommendations for engaging patients in value assessment in its 2016 paper, “A Roadmap to Increased Patient Engagement in Value Assessment.”

The objective of this analysis was to better understand the extent to which ICER meaningfully engages patients and other stakeholders throughout its public comment process.

To examine this issue more closely, one aspect of the ICER stakeholder engagement process— public comment letters to draft evidence reports— was considered. These were compared to ICER’s final value assessment reports and analyzed to determine the extent to which ICER has incorporated input from patients and other stakeholders.

- This analysis examines the public comment process relied upon by ICER, a private, non-profit organization whose assessments are currently being utilized by both private and public payers.
- The objectives of this analysis included:
 - Identification of areas of particular importance to stakeholders
 - Quantitatively capturing the extent to which stakeholder comments are incorporated in ICER’s ongoing work
- Based on findings, we discuss several ways ICER can engage with stakeholders to ensure their input is meaningfully incorporated into assessments.

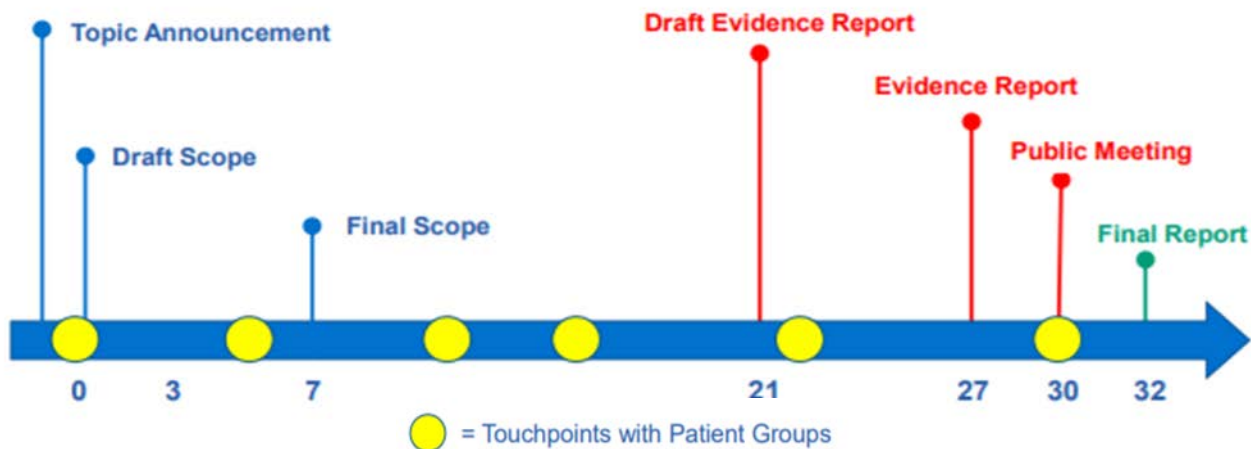
* This assessment only looks at patient centeredness in value assessment from a quantitative analysis of response to and incorporation of input; it does not examine other factors such as whether outcomes that matter to patients are consistently included and appropriately weighted in the assessments.

ICER's open comment period has evolved to increasingly elicit and acknowledge more patient input.

The figure below shows the formal ICER touchpoints with patient groups.

- Prior to 2017, ICER provided responses to stakeholder comments in a **letter format**. However, this resulted in many comments from stakeholders not being addressed.
- Starting in 2017, ICER adopted a new **table response format**, which allows ICER to acknowledge all stakeholder comments received.

Touchpoints with Stakeholders During ICER Value Assessment Process



Certain ICER reports were chosen for evaluation because of their broad population impact and number of stakeholder comments.

ICER Final Evaluation Reports Included in Analysis:

Non-Small Cell Lung
Cancer

November 1, 2016
122 comments

Osteoporosis

July 19, 2017
163 comments

Ovarian Cancer

September 28, 2017
62 comments

Migraine

July 5, 2018
113 comments

- Selected reports include a **diverse range of stakeholders**, with significant submissions from patient advocates, industry, and professional societies.
- ICER evaluations **published between 2016 and 2018** were conducted over a period of time during which ICER **refined their process** for acknowledging stakeholder comments.

This analysis quantified how ICER acknowledges and incorporates external feedback into its final value assessment reports.

Definitions

- **Acknowledgement of Comment:** ICER provided a written response to a stakeholder's comment in their published and publicly-available "Response to Comments"
 - Quality of ICER's response to comments was evaluated by identifying **acknowledgment** of the comment by ICER to determine if ICER replied to the comment
- **Incorporation of Comment:** ICER modified final evidence report to reflect the stakeholder's suggestion
 - ICER's response to comments was subsequently evaluated by identifying **incorporation** of the comment by ICER to determine if ICER utilized any comment recommendations in future work
- **Recommended Solution:** In addition to providing comments on draft reports, stakeholders proactively included suggested solutions for ICER to consider in addressing their comments
 - Comments from stakeholders were evaluated by identifying **presence or absence of a recommended solution** for the comment provided and whether it was incorporated

Comments were evaluated by categorizing and accounting for specific attributes.

Stakeholder comments were assessed by evaluating comment details, including the type of comment, comment category, and if solutions were recommended by stakeholders.

When Xcenda evaluated stakeholder comments, the following aspects were taken into consideration:

Stakeholder Type	<i>One of the following:</i> <ul style="list-style-type: none"> • Industry • Patient advocates • Professional/provider society
Stakeholder Name	<i>Name of stakeholder submitting comment</i>
Comment Category	<i>Comments were placed into 1 of the listed categories</i>
Type of Comment	<i>One of the following:</i> <ul style="list-style-type: none"> • General feedback: Comments related to ICER's value framework • Methodological: Comments related to specific methods used for a single evaluation
Comment by Stakeholder	<i>Actual comment provided</i>
Solution Provided by Commenter?	<i>One of the following:</i> <ul style="list-style-type: none"> • Yes • No
Solution	<i>Actual solution provided</i>

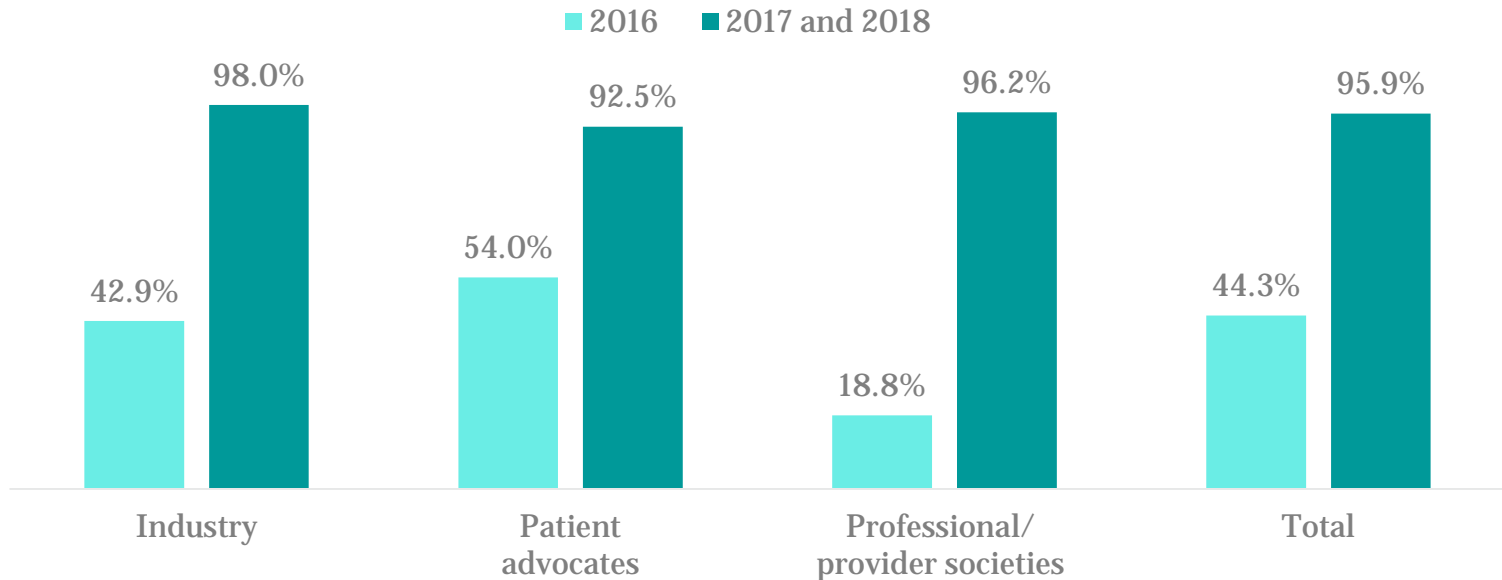
Adequacy of existing evidence
 Assumptions
 Budget impact
 Clinical expertise
 Comparators
 Cost offsets
 Data accuracy/consistency
 Definition of value
 Disease burden
 Efficacy data
 Impact on innovation
 Interventions
 Non-FDA-approved treatments and dosages
 Network meta-analysis
 Model inputs
 Model perspective
 Patient perspective
 Patient population/subpopulation
 Patients' access to treatment options
 Pricing considerations
 Quality-adjusted life-years
 Safety data
 Sensitivity/scenario analyses
 Time horizon
 Transparency comments
 Transparency methods
 Unstated limitations
 Utility data

Findings & Results

Since refining its process for public commenting in 2017, ICER has acknowledged more than 95 percent of comments received from stakeholders.

Since 2017, ICER has provided a written response to a majority of comments received, explaining why feedback was or was not incorporated.

Percentage of Total Stakeholder Comments Acknowledged by ICER



Total comments

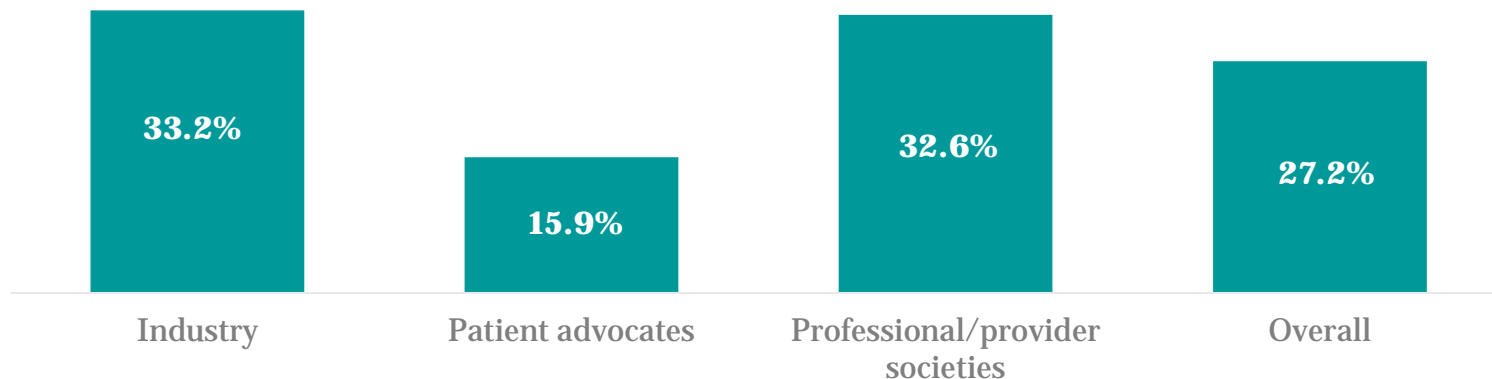
2016: Industry, N=56; patient advocates, N=50; professional/provider societies, N=16

2017 and 2018: Industry, N=152; patient advocates, N=107; professional/provider societies, N=79

Despite ICER acknowledging a majority of comments, only 27 percent were incorporated into final reports.

Comments from patient advocates were half as likely to be incorporated compared to other stakeholder groups.

Percentage of Stakeholder Comments Incorporated Into ICER Final Evidence Reports

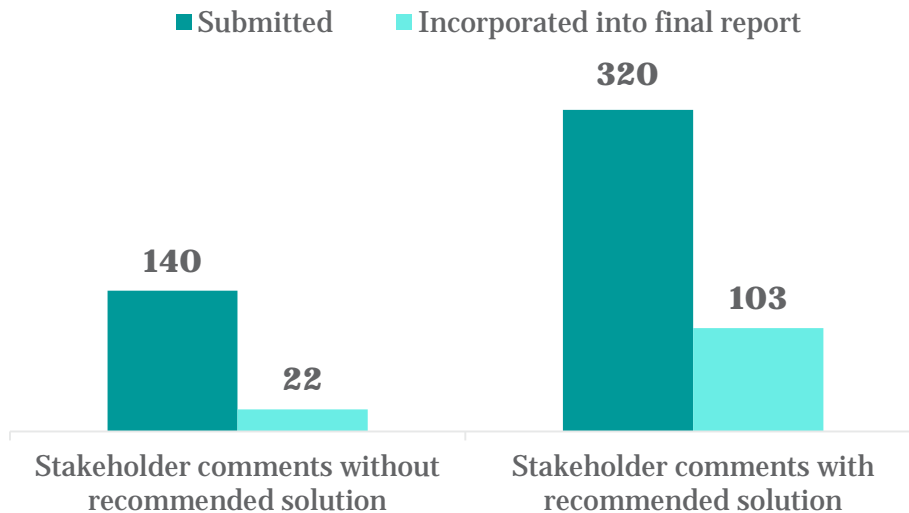


All comments: Industry, N=208; patient advocates, N=157; professional/provider societies, N=95

Even when stakeholders provided proposed solutions to address their comments, ICER incorporated only one third of such comments.

About one third (32 percent) of stakeholder comments that included a recommended solution were incorporated into ICER's final evidence reports, while approximately 16 percent of those that did not were incorporated.

ICER's Incorporation of Results by Presence of Recommended Solutions (Number of Comments)



“ICER’s value framework does not include consideration of low-grade, chronic side effects... Given the body of evidence currently available on long-term effects of the vast majority of the “prevailing standard of care”, CSC **strongly encourages ICER to incorporate that information as an important component in the calculation of clinical-effectiveness.**”

-Cancer Support Community ¹

Solution provided: Industry, N=191; patient advocates, N=75; professional/provider societies, N=54; total, N=320

Solution not provided: Industry, N=17; patient advocates, N=82; professional/provider societies, N=41; total, N=140

1. ICER. Non-small cell lung cancer: Public Comments. <https://icer-review.org/material/nsclc-public-comments>.

Stakeholders most frequently commented on efficacy data and patient population considerations.

Percent of Total Comments



“The emphasis on ‘therapeutic gain’ values from placebo-controlled trials **may lead to underestimation of efficacy**... “therapeutic gains” to extrapolate the clinical impact of an active intervention has **severe limitations.**”

-American Headache Society and American Migraine Foundation¹

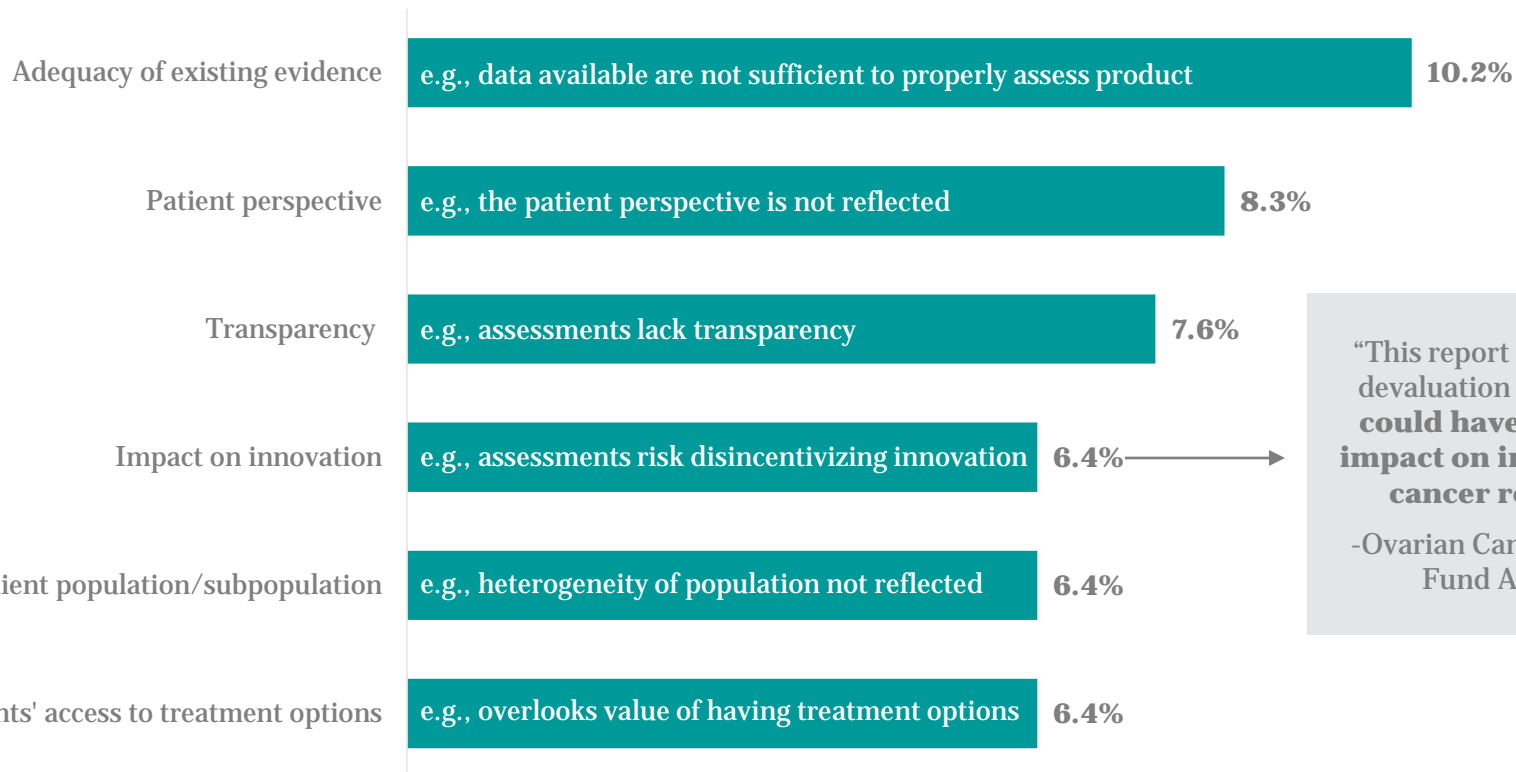
“Treatment discontinuation is one of the variables impacting cost-effectiveness outcomes... **However, the estimated results lack face validity**... The ICER analysis should ensure that the discontinuation rate estimates are appropriately derived and used in the analysis.”

-Teva¹

1. ICER. Migraine: Public Comments. <https://icer-review.org/material/cgrp-response-to-comments/>

Approximately 10 percent of comments from patient advocates were related to the adequacy of existing evidence.

Percentage of Comments From Patient Advocates for Each Comment Category



“This report may lead to a devaluation of PARP and could have a negative impact on innovation in cancer research.”
 -Ovarian Cancer Research Fund Alliance ¹

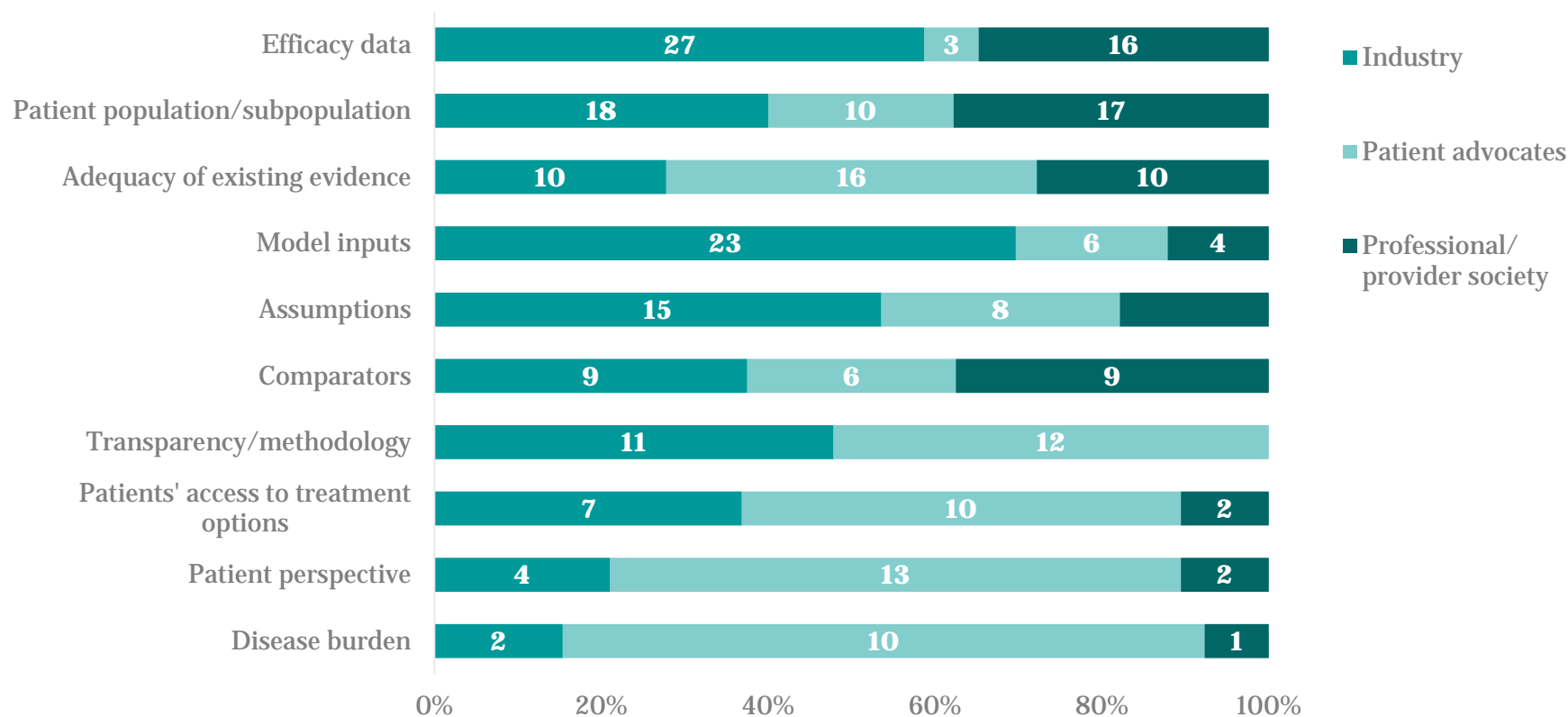
Patient Advocates Comments: N=157

Graph displays top 6 comment categories from patient advocate stakeholders, with examples of potential types of comments received for each category. Examples listed are not direct quotes from any stakeholders.

1. ICER. Ovarian cancer: Public Comments. <https://icer-review.org/material/ovarian-cancer-public-comments/>

Patient advocates were the driving force behind comments on patient perspective and disease burden.

Comments Contributed by Stakeholder, Number of Comments and Percent of Total Category



Displaying top 10 categories, full list of categories included in appendix

Patient advocates most frequently commented on the adequacy of existing evidence, patient perspective, and transparency.

Adequacy of Existing Evidence

“Lack of long-term data should not justify undervaluing new migraine therapies. We are concerned that a premature assessment based on inadequate evidence could result in delayed treatment access for migraine patients who have already waited years for a viable therapy.”

- Headache and Migraine Policy Forum ¹

Patient Perspective

“Patients must have a meaningful role in the discussion of value given that they are directly impacted by a report that seeks to define the effectiveness and value of their treatment options. Therefore, accounting for how patients define the value of their treatment options should be critical to ICER’s analysis.”

- Aimed Alliance ²

Transparency

“[We] respect the proprietary nature of the effort; however, the lack of transparency calls into question its validity... Furthermore, there needs to be transparency about the expert clinicians who are advising on the real-world use of the therapies, the model inputs, and how the model will be used.”

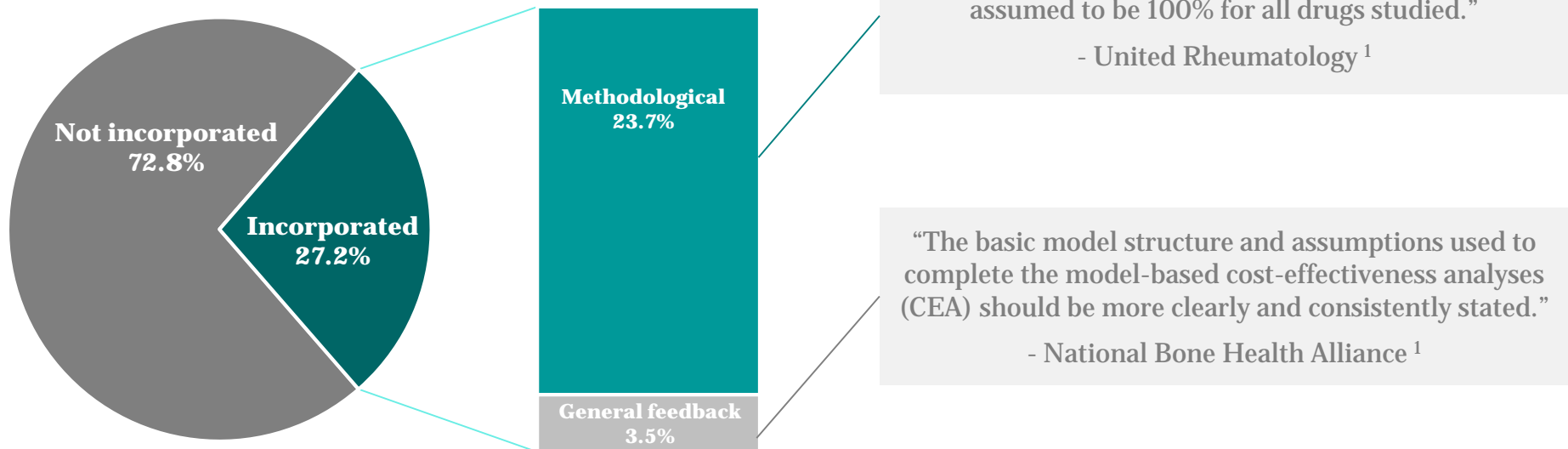
- LUNgevity Foundation ³

1. ICER. Migraine: Public Comments. <https://icer-review.org/material/cgrp-response-to-comments/>
2. ICER. Osteoporosis: Public Comments. <https://icer-review.org/material/osteo-draft-report-comment/>
3. ICER. Non-small cell lung cancer: Public Comments. <https://icer-review.org/material/nscl-public-comments>

ICER was more likely to incorporate feedback related to methodology than general feedback on their framework.

Comments related to methodology accounted for 73 percent of all comments *submitted* but 87 percent of comments that were *incorporated* into future work.

Stakeholder Comments Incorporated Into ICER Final Evidence Reports



All comments: Industry, N=208; patient advocates, N=157; professional/provider societies, N=95

1. ICER. Osteoporosis: Public Comments. <https://icer-review.org/material/osteo-draft-report-comment/>

Evaluation of stakeholder input to ICER also took into account how ICER addressed each comment.

ICER's response to stakeholder comments were assessed by evaluating the extent to which comments were acknowledged and incorporated into future work by ICER.

The evaluation of ICER's response to comments considered the following:

Comment Acknowledged by ICER?	<i>Determine if ICER replied to stakeholder comment</i> <i>One of the following:</i> <ul style="list-style-type: none">• Yes• No
Response by ICER	<i>Actual comment provided</i>
Solution Incorporated Into Report?	<i>Determine if ICER utilized any comment recommendations for future ICER work</i> <i>One of the following:</i> <ul style="list-style-type: none">• Yes• No
Strength of Response	<i>Scored by following criteria</i>

A method was developed to estimate a quantitative score for ICER's response to stakeholder comments.

If ICER provided response but **did not** incorporate comment:

Criteria	Score	Total Possible Points
Provides response	1	1
Partially addresses comment <i>OR</i> Fully addresses comment	0.5 1	1
States comment is beyond scope of report <i>OR</i> Provides relevant rationale why no further action was taken	0.5 1	1
Provides relevant example partially illustrating how comment is already accounted for in report <i>OR</i> Provides relevant example fully illustrating how comment is already accounted for in report	0.5 1	1

If ICER provided response and **did** incorporate comment:

Criteria	Score	Total Possible Points
Provides response	1	1
Partially addresses comment <i>OR</i> Fully addresses comment	0.5 1	1
States comment was taken into consideration for inclusion in final report <i>OR</i> Provides relevant rationale why further action was taken	0.5 1	1
Takes further action to partially incorporate comment recommendation <i>OR</i> Takes further action to incorporate all feedback received	0.5 1	1

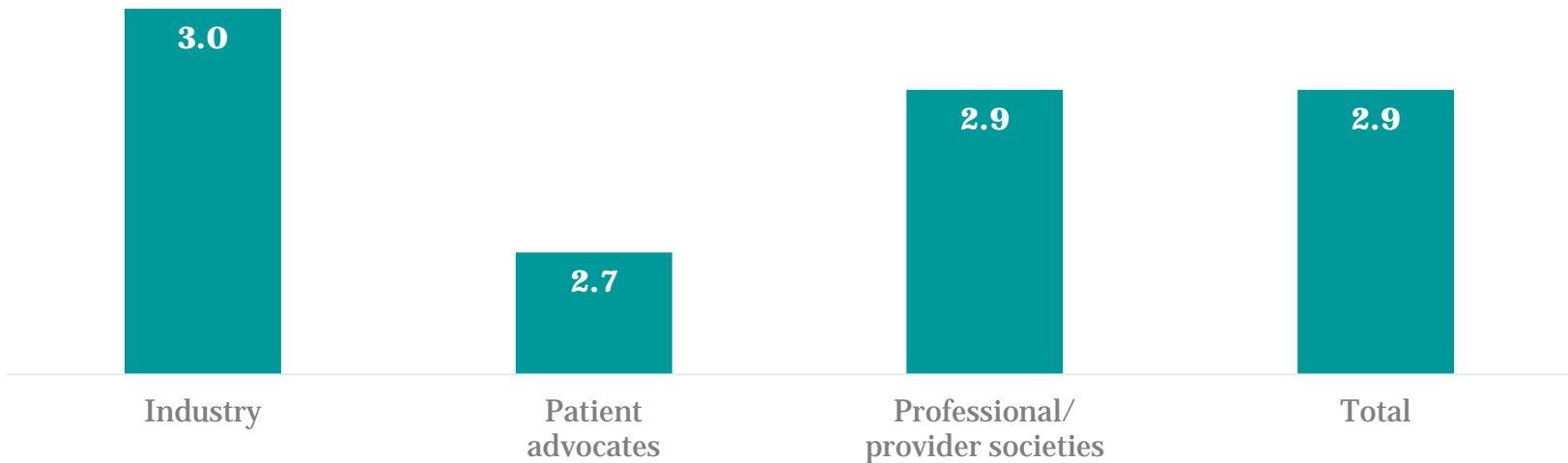
0.00–0.49 = N/A
0.50–0.99 = Very low
1.00–1.49 = Low
1.50–1.99 = Low-moderate

2.00–2.49 = Moderate
2.50–2.99 = Moderate-high
3.00–3.49 = High
3.50–4.00 = Very high

ICER provided the least robust responses to comments submitted by patient advocates.

Comments from professional/ provider societies and industry stakeholders received the most robust responses.

**Average Score of ICER Responses
(Potential Range 0 – 4)**



All comments: Industry, N=208; patient advocates, N=157; professional/provider societies, N=95

0.00–0.49 = N/A
0.50–0.99 = Very low
1.00–1.49 = Low
1.50–1.99 = Low-moderate

2.00–2.49 = Moderate
2.50–2.99 = Moderate-high
3.00–3.49 = High
3.50–4.00 = Very high

Summary of Key Findings & Recommendations

Summary of Key Findings

- Since refining its process for public commenting in 2017, ICER has acknowledged more than 95 percent of comments received from stakeholders.
- Even when stakeholders provided proposed solutions to address their comments, ICER incorporated only one third (32 percent) of such comments.
- Comments from patient advocates were least likely to be acknowledged and incorporated (15.9 percent) compared to industry (33.2 percent) and professional/provider societies (32.6 percent).
- Patient advocates most frequently commented on adequacy of existing evidence, patient perspective, and transparency.
- ICER was more likely to incorporate input on methodology than general feedback on their framework.
- ICER was least likely to provide a robust response to comments submitted by patient advocates.

PIPC Recommendations for Meaningful Incorporation of Patient Feedback into Value Frameworks

- To achieve a truly patient-centered value assessment, it is imperative that patient advocates' insight and expertise are meaningfully considered and incorporated into frameworks.
- ICER and other value assessment organizations should utilize tools, such as the National Health Council's Patient-Centered Value Model Rubric, to better understand what methodologies and processes can be undertaken to ensure a value assessment is truly patient-centered.
- Several ways in which ICER can improve its engagement with patients and optimize its public comment period include:
 - Proactively engage patients and patient advocacy groups in the model development process
 - Present a layman's version of the model specifications and analysis plan so patient advocates can understand what is being proposed and comment accordingly
 - In evaluations where the model is made available to manufacturers, models should also be available to patient advocates in an easy-to-understand format
 - Permit sufficient time for review of all of the above

Where PIPC Stands: Achieving Patient-Centeredness in Value Frameworks

Process:

- Develop a transparent process through which frameworks are developed, implemented, and validated
- Meaningfully engage with patients and organizations representing the impacted patient communities and clinical experts in the specific treatment area under consideration

Standards:

- Rely on a range of rigorously developed evidence that fully capture value that matters to patients
- Quantify value in a way that incorporates outcomes that are meaningful to patients and people with disabilities
- Address costs holistically, including costs that matter to patients, such as long-term, personal and societal costs
- Support shared decision making, which is foundational to advancing value in ways that are patient-centered

Safeguards:

- Acknowledge diversity and differences among patients, and avoid the 'one-size-fits-all' mentality of value
- Preserve protections in the Affordable Care Act against use of comparative or cost-effectiveness research to make centralized value judgements