
# Elevating the Patient Voice in Health Economic Assessments Survey Report

**October 2022**

# EXECUTIVE SUMMARY

The Lupus and Allied Diseases Association (LADA) was founded in 1978 in Utica, New York and is dedicated to improving access to care and quality of life for those affected by lupus and allied diseases by fostering collaboration among stakeholders, promoting unity in the community, and wielding the patient voice as a catalyst to advance innovative advocacy, education, awareness and biomedical research initiatives. As a national all-volunteer organization led by people with lupus and their loved ones, we work to ensure that the patient stakeholder is included as an equal participant in the healthcare, regulatory and public policy arenas and across the research continuum.

As physicians, patients, advocates, health plans, drug manufacturers, and policymakers search for tangible solutions to address increasing healthcare costs and access issues in the United States, Institute for Clinical and Economic Research (ICER) drug reviews and other health economic assessments (HEAs) and value discussions have become commonplace today. For purposes of this survey, HEAs represent Health Technology Assessments (HTAs) and Value Assessments (for example ICER Drug Reviews).

LADA actively engaged and participated in a HEA through ICER for Lupus Nephritis (LN) therapies over an 8-month period during 2020-2021. By drawing on our own unique experiences in using the patient and caregiver voice to drive the review to assess the drugs' worth based on what people living with lupus and lupus nephritis and their caregivers value most in a treatment, we were able to effectively contribute to the assessment. The involvement of LADA and other lupus community participants and advocacy stakeholders ensured that the patient and caregiver viewpoints were properly accrued and accounted for in the lupus nephritis drug evaluation. In this case, the process yielded a positive assessment of both drugs being appraised, which has not been the usual outcome of these reviews. We would like to note that although the review was positive, it has not resulted in the lupus community gaining access to both treatments.

As a takeaway from this experience, LADA sought to better understand the collective patient advocacy experience in HEAs in order to demystify the process and encourage and prepare other patient advocacy organizations in different disease states to strategically engage and empower their own communities to effectively participate. LADA developed and conducted a survey between February and April 2022 to gauge the knowledge and experience of U.S. based patient advocacy organizations concerning HEAs and to inform the degree and structure of potential educational resources needed to better prepare groups to participate. The survey was disseminated to a diverse group of advocacy stakeholders throughout the United States resulting in a total of 112 survey respondents.

The survey data from duplicate and incomplete responses and non-patient organization respondents were not included in the analysis for this report, reducing the number of complete survey respondents to 96. The data collected in the survey reflect a wide array of advocacy stakeholder experiences with HEAs from large national umbrella and chronic disease foundations to small grassroots rare disease advocacy organizations and reveal that there is willingness among patient advocacy organizations to be an important participant in HEA processes. The

survey findings also demonstrate the need for additional resources in order for patient advocacy organizations to be able to feel informed and prepared enough to actively participate in these assessments.

Survey participants began the assessment by responding to several identifier questions including name, organization name, and e-mail (to be used only for communication purposes and to eliminate duplicates and non-patient organizations) and then moved on to general questions about their organization. Respondents then followed one of two tracks based on their response to Question 5 asking whether they had participated in HEAs or ICER Reviews. Those that responded YES (N=41) were then asked an additional seven questions (6A-12A), while those that responded NO (N=55) were asked an additional three (6B-8B) questions.

Among the key learnings from those patient advocacy organizations that participated in HEAs was that the participation and contributions of actual patient and caregiver stakeholders is of paramount importance during HEAs with over 73% of groups including them in their processes. Almost 43% of respondents identified community roundtables or forums as the most effective means for collecting this type of feedback and 34% chose surveys as the best collection method. While it is assumed that the outcomes of HEAs are either positive or negative, 46% of advocacy groups who participated in HEAs indicated that their view of the final result of the assessment was neither positive nor negative for their community.

The most surprising survey findings from the patient advocacy organizations that participated in HEAs came from the those who responded that the HEA result for their community was positive, but 64% of them then indicated that their community did not benefit from the assessment by gaining access to the new treatment. For these groups, 89% responded that they were engaging with drug manufacturers and payers to try to improve access to the treatment. Also unexpected was the discovery that despite the outcome of the assessment, 63% of patient advocacy organizations that participated in HEAs responded that the overall participation experience was positive, demonstrating that they are open to being engaged and sharing their community perspectives.

For those patient advocacy organizations that did not participate in HEAs, 69% identified not being invited as the primary reason for not participating, while 37% chose lack of awareness as the reason. In addition, respondents selected limited staff, financial resources and time, as well as lack of experience/expertise as reasons for not participating. One of the most interesting survey finding from the non-participating groups was that 72% of them indicated that they would engage if they understood how to effectively participate through educational forums, had adequate resources, and were able to connect with organizations who had participated in HEAs. Of those three resources, 93% of respondents chose connecting with other advocacy organizations who had participated in HEAs as the most effective tool to help them to participate in future assessments.

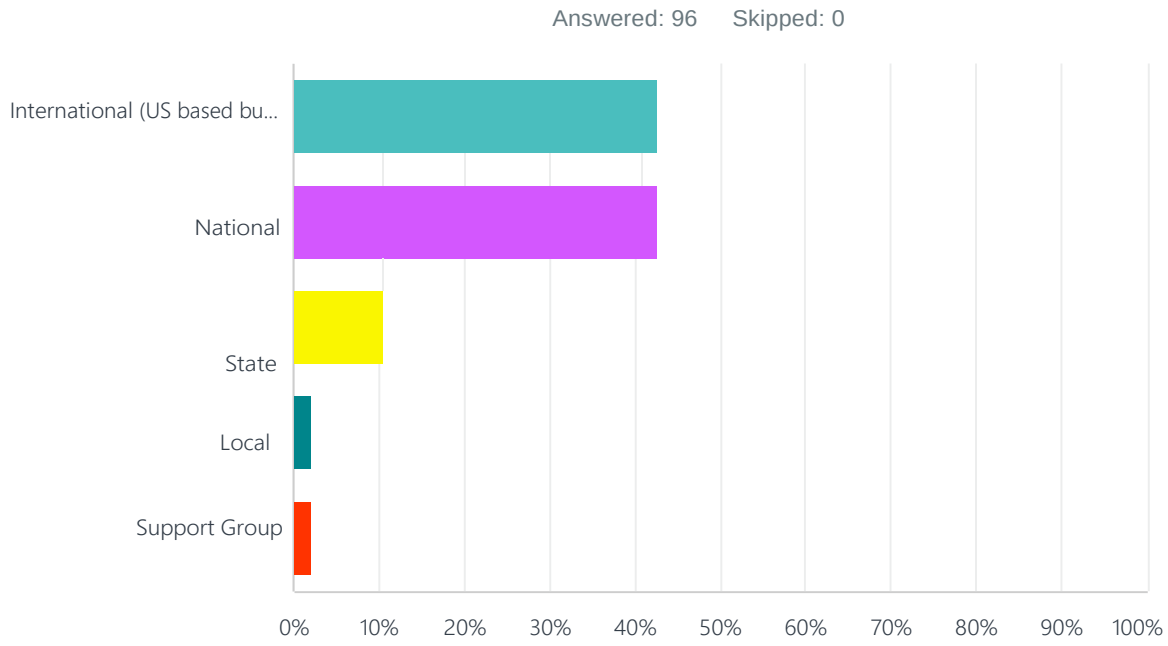
The results of this groundbreaking Health Economic Assessments Participation Survey for U.S. Patient Advocacy Organizations yields significant learnings for the broader advocacy and value assessment community while underlining potential opportunities for the LADA Team to further investigate and address. The findings also provide evidence to support our fundamental belief in the importance of patient and caregiver stakeholders and their voices being included in HEA processes and that this conviction is also shared by many in the patient advocacy community.

LADA is dedicated to improving the lives of people affected by lupus and allied diseases and other rare and chronic conditions of unmet need and we believe that this report represents a significant step towards achieving that goal by highlighting the HEA experiences of U.S based patient advocacy organizations and helping to identify what resources are needed to empower groups to more effectively engage and participate in HEAs and ICER Reviews.

We would like to recognize and thank the various stakeholders who helped to make this pioneering initiative possible. We extend sincere appreciation to the individuals, patient and healthcare provider organizations, coalitions, research entities, biopharmaceutical companies and other entities that helped to share and promote the survey, and, above all, the many patient advocacy organizations who took the time to participate in this brief survey. We gratefully acknowledge our pharmaceutical sponsors for their generous support in providing funding for this initiative, and the LADA Team for their contributions and sole management of the project.

# LADA Health Economic Assessments Survey Summary

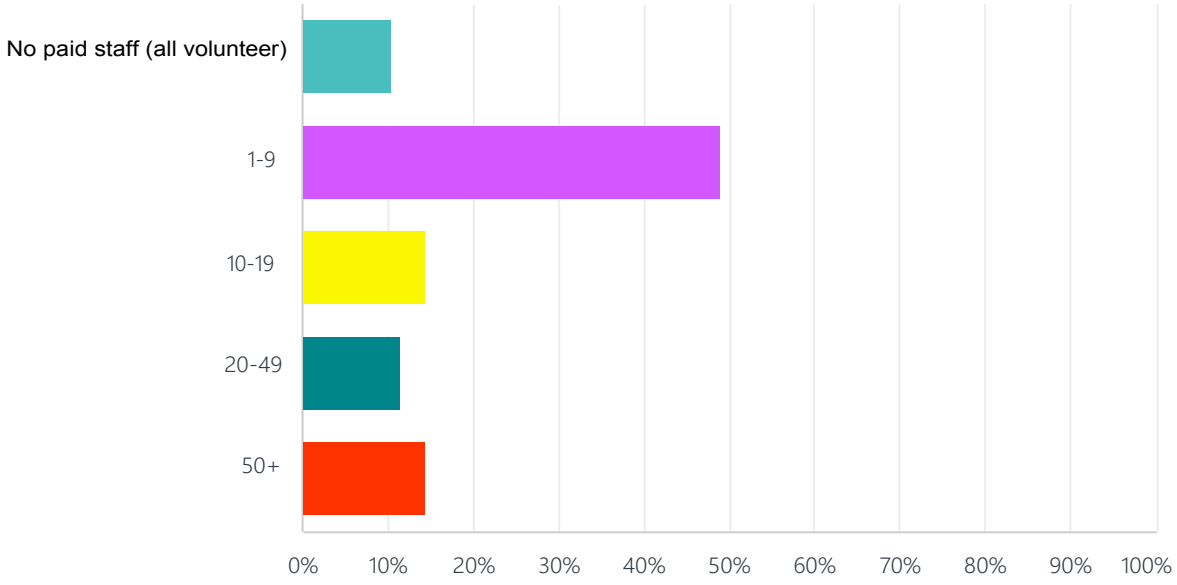
## Q1 Is your organization (select one)




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| International (US based but Global reach) | 42.71% | 41 |
| National | 41.67% | 40 |
| State | 11.46% | 11 |
| Local | 2.08% | 2 |
| Support Group | 2.08% | 2 |
| **TOTAL** | | **96** |

# Q2 What is the number of paid staff in your organization?

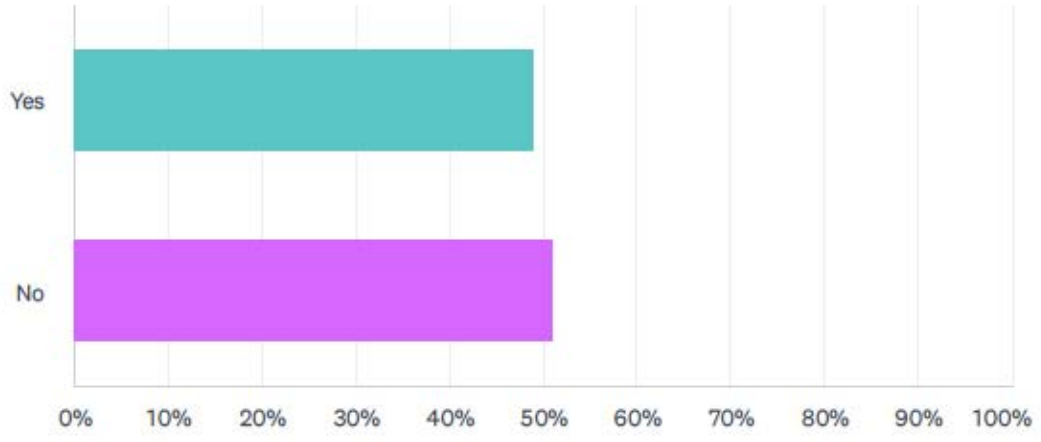
Answered: 96 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| No paid staff (all-volunteer) | 10.42% | 10 |
| 1-9 | 48.96% | 47 |
| 10-19 | 14.58% | 14 |
| 20-49 | 11.46% | 11 |
| 50+ | 14.58% | 14 |
| **TOTAL** | | **96** |

# Q3 Does your organization represent a Rare Disease (less than 200,000 people diagnosed)?

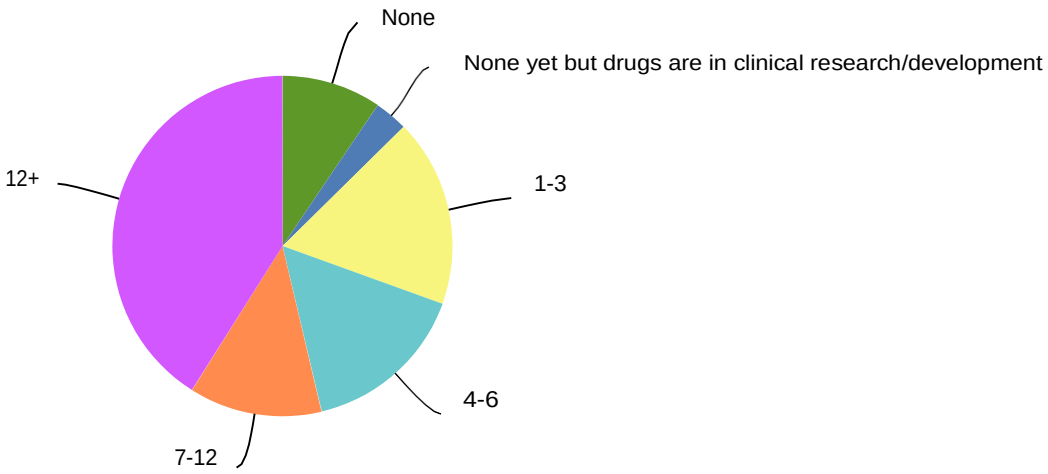
Answered: 96 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Yes | 48.96% | 47 |
| No | 51.04% | 49 |
| TOTAL | | 96 |

# Q4 How many FDA-approved treatments currently exist for the disease/condition you represent?

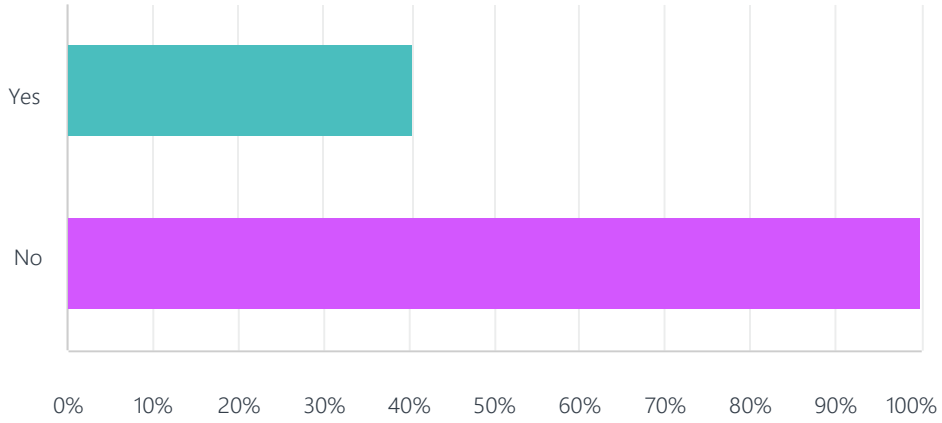
Answered: 95 Skipped: 1




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| None | 9.47% | 9 |
| None yet but (drugs are in clinical research/development) | 3.16% | 3 |
| 1-3 | 17.89% | 17 |
| 4-6 | 15.79% | 15 |
| 7-12 | 12.63% | 12 |
| 12+ | 41.05% | 39 |
| **TOTAL** | | **95** |

# Q5 Has your organization participated in an ICER Drug Review or other Health Economic Assessment (HEA) or provided written comments?

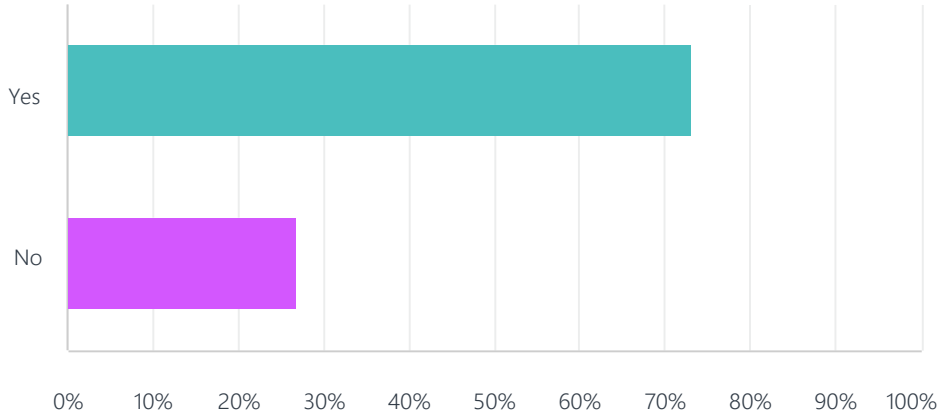
Answered: 96 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Yes | 41.67% | 41 |
| No | 58.33% | 55 |
| **TOTAL** | | **96** |

# Q6A Did you engage actual patients and/or caregivers to work with you during the assessment? (HEA Participating Groups only)

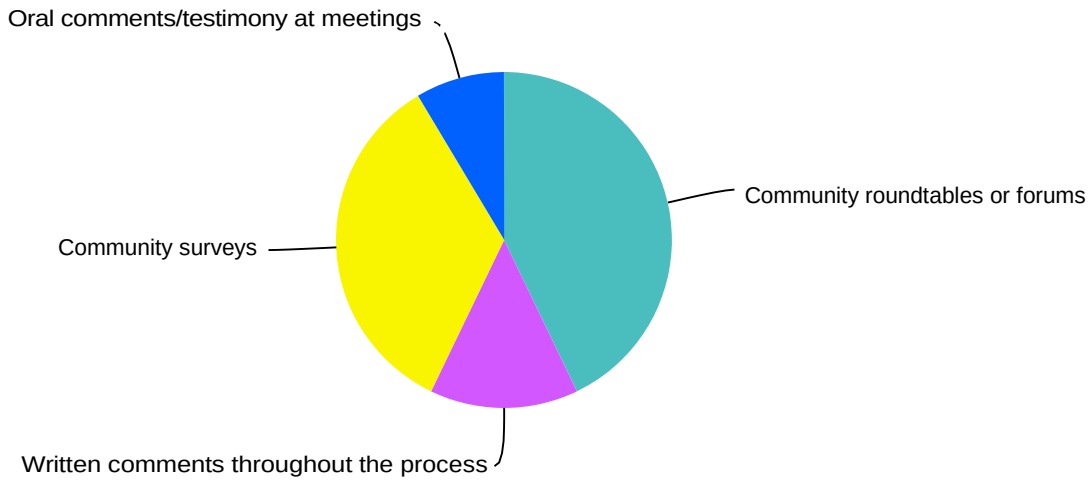
Answered: 41 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Yes | 73.17% | 30 |
| No | 26.83% | 11 |
| **TOTAL** | | **41** |

# Q7A What do you believe is the most effective collection method to capture patient and/or caregiver input in HEAs? (HEA Participating Groups only)

Answered: 35 Skipped: 6

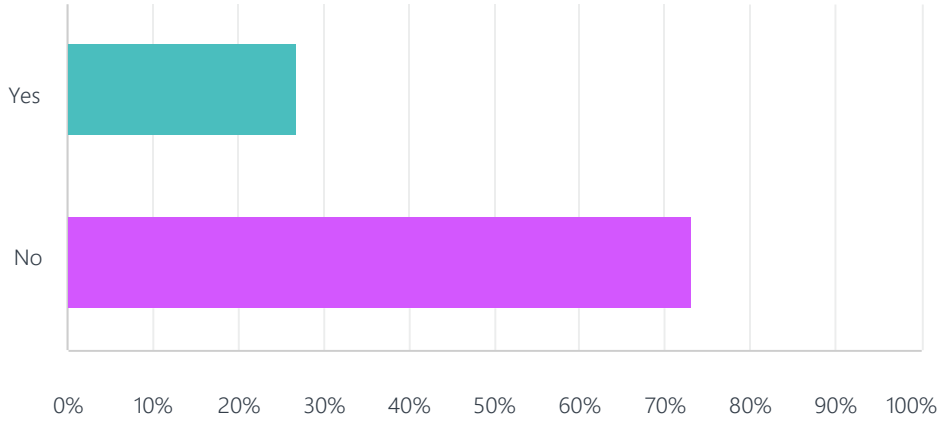



| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Community roundtables or forums | 42.86% | 15 |
| Written comments throughout the process | 14.29% | 5 |
| Community surveys | 34.29% | 12 |
| Oral comments/testimony at meetings | 8.57% | 3 |
| **TOTAL** | | **35** |

| OTHER (PLEASE SPECIFY) |
|---|
| Surveys can capture input from multiple perspectives vs. one representative |
| All of the above |
| I don't believe there is a most effective collection method. Patients and caregivers are diverse, and there should be diverse collection methods for them to share their perspectives. |
| Multiple different methods, not just one |
| A combination of tactics |
| I think it needs to be a combination, surveys are a good broad perspective that can help direct content/topics to be further probed in smaller focus group type discussions |
| All of the above - patients/providers/caregivers need to be represented throughout the whole process |
| Data is so important, but very important to have patients be involved with initial conversations with ICER and throughout the process |
| A combination of the above |
| 1) patient-centered outcomes measured in pivotal trials; 2) Longitudinal studies using patient |

# Q8A Did you hire an outside consultant such as an economist to assist you during the assessment? (HEA Participating Groups only)

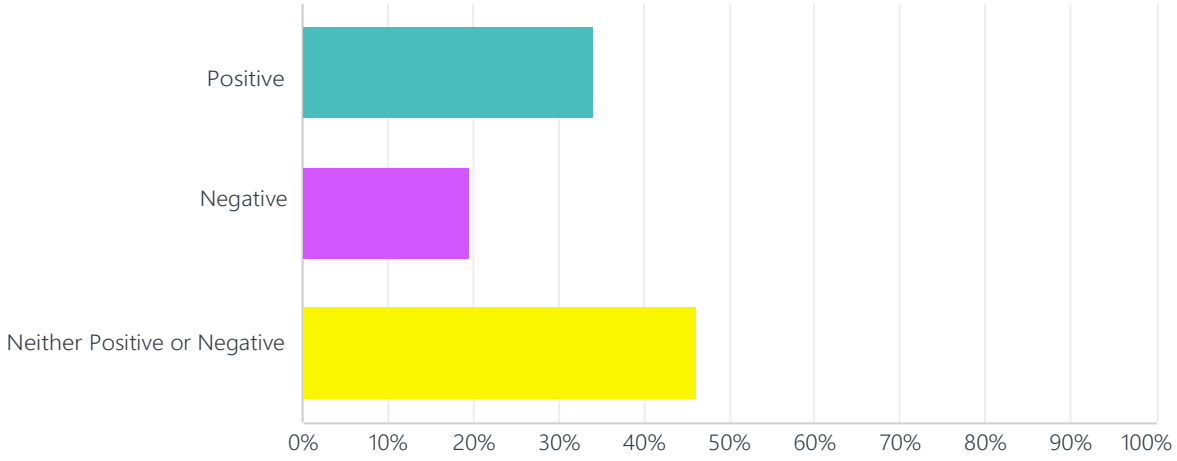
Answered: 41 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | |
|---|---|---|
| Yes | 26.83% | 11 |
| No | 73.17% | 30 |
| **TOTAL** | | **41** |

# Q9A What was the final result of the assessment for your community? (HEA Participating Groups only)

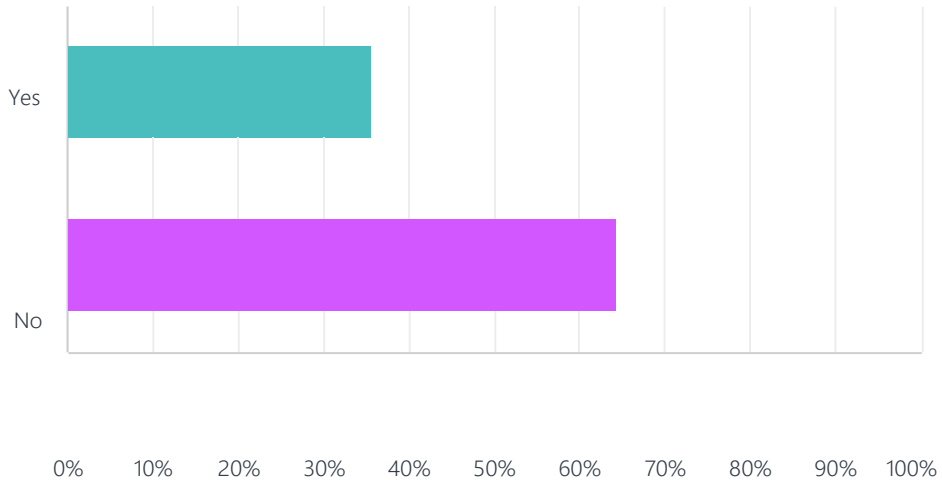
Answered: 41 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Positive | 34.15% | 14 |
| Negative | 19.51% | 8 |
| Neither Positive or Negative | 46.34% | 19 |
| **TOTAL** | | **41** |

# Q10A If positive, did your community actually benefit from the assessment by gaining access to the treatment through formulary coverage, fair pricing, and reasonable patient cost sharing? (HEA Participating Groups only)

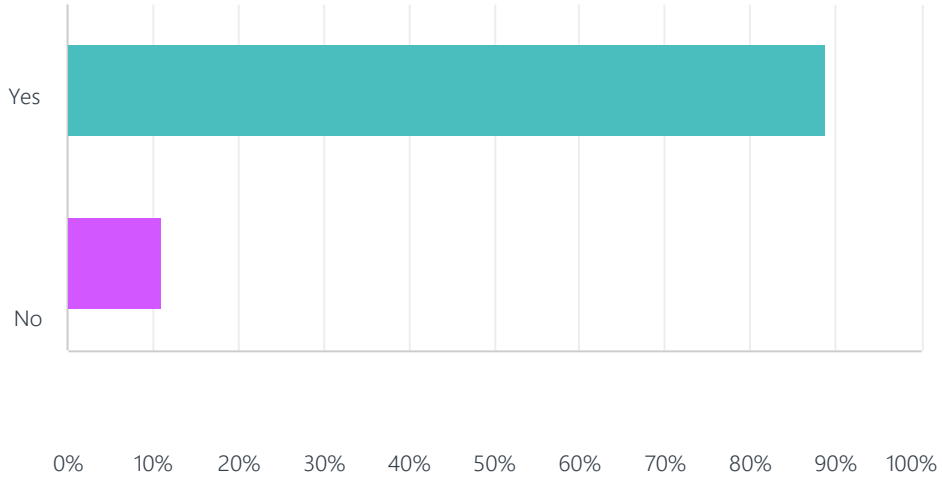
Answered: 14 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Yes | 35.71% | 5 |
| No | 64.29% | 9 |
| TOTAL | | 14 |

## Q11A If no, are you engaging with drug manufacturers and payers to try to improve access? (HEA Participating Groups only)

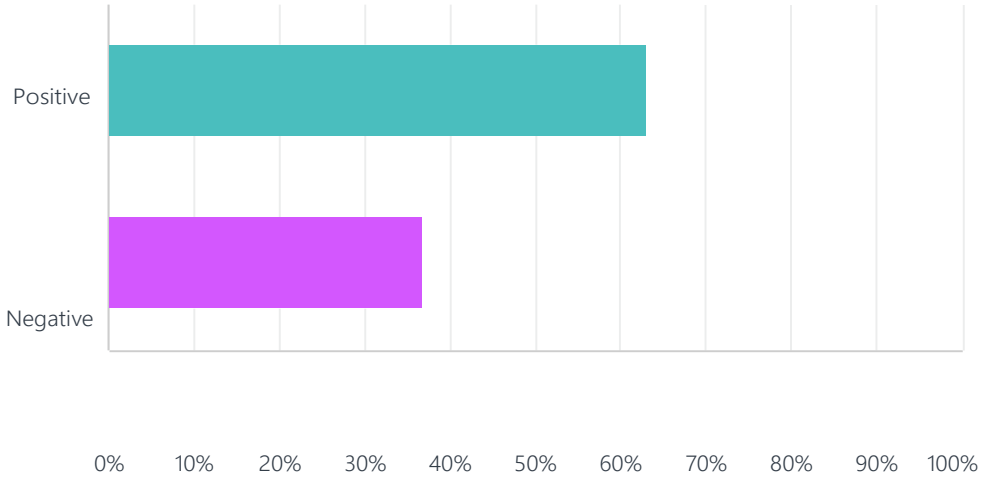
Answered: 9 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Yes | 88.89% | 8 |
| No | 11.11% | 1 |
| **TOTAL** | | **9** |

# Q12A Aside from the end result of the review, how did you find the overall experience of participating in the assessment? (HEA Participating Groups only)

Answered: 38 Skipped: 3

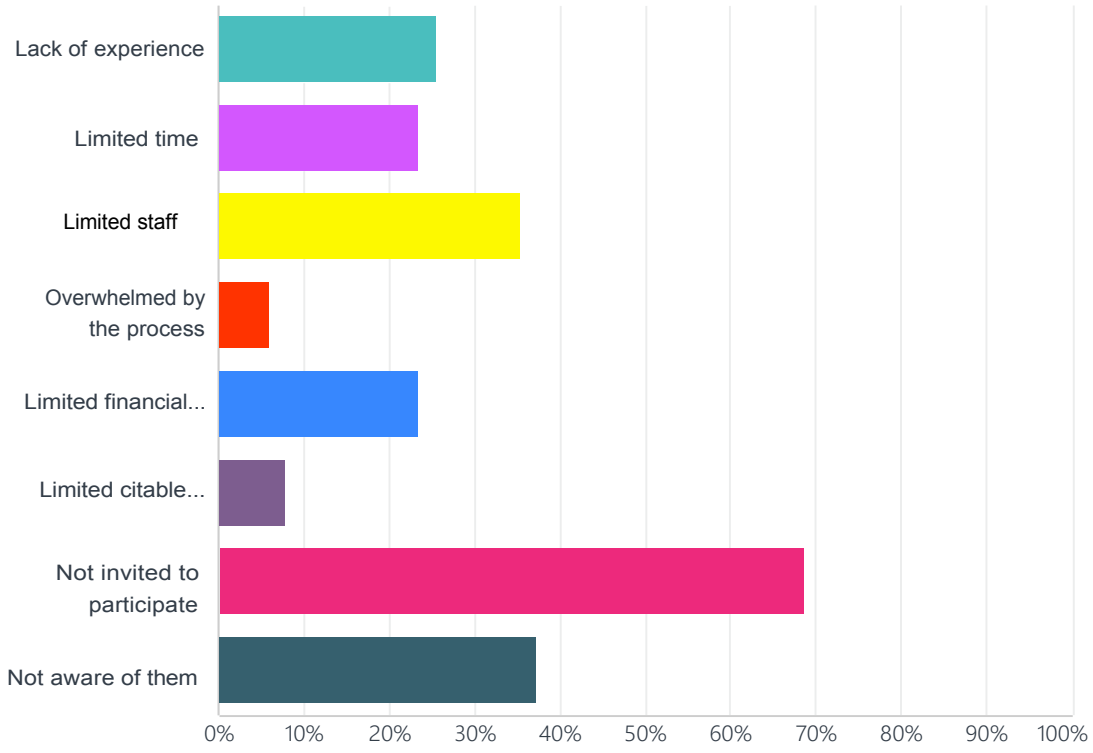



| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Positive | 63.16% | 24 |
| Negative | 36.84% | 14 |
| **TOTAL** | | **38** |

| PLEASE EXPLAIN YOUR RESPONSE CHOICE FOR QUESTION 12A |
| --- |
| It was a great deal of work, however we are able to be better advocates for our community because of our participation. Overall I think our participation in the ICER review helped to educate us on HEA and propel other work in this area forward, however we are very disappointed with the final results from the review |
| It felt like lip service. Our comments and concerns were not taken into account and the patient perspective was dismissed. This was early on in the process, but it was not a good experience. |
| The Review team listened and considered the evidence we provided and made modifications to its assessment base on BWHI findings. |
| Always good to have more exposure and awareness. |
| The team conducting the review was thorough. |
| No feedback or acknowledgement that ICER considered or reflected our input in its decision. |
| Neither |
| If there was a neutral category, I would have chosen that. Overall, it's a lot of work and takes considerable time. For our community, the end results did not have an impact one way or the other. I do think our involvement has improved the overall process. |
| It was important to have a voice and speak our truth. |
| We had collaborative resources. |
| I had the opportunity to be present and explain the rationale for approving the use of a cancer therapy. Being present gave me a better insight on the process, who was in the room and was I able to watch how the assessment team presented their findings. The importance of attending also benefited the end result. They were able to ask questions of the advocates and we were able to ask them questions. |
| We learned a great deal that will help us to be more effective in drug research and development and our advocacy efforts. |
| Helped inform on the part of the process post-approval. |
| JDRF has experiencing conducting HEA to support adoption of innovative therapies. These have been positive as we ensured our community was engaged. Perhaps less positive experience in those HEA we did not lead. |
| We felt there were many inaccuracies in their report and had to heavily invest our resources to ensure the data and information was accurate. |
| It felt like a sham proceeding. Comments were solicited, but carried no weight in the decision making process. |
| ICER was very engaged with us, though there were ways they could have had better patient representation at the meeting itself. |
| We tried our hardest to advocate for the Cystic Fibrosis community, but it felt like they had their own agenda and nothing we said could move them. In retrospect, I almost wish I didn't participate. |
| Confusing process |
| ICER showed an interest to learn more about MG through patient and physician engagement and demonstrated a collaborative approach with MGFA. |
| For migraine patients, many of whom are disabled by the disease, the public input process was confusing and inaccessible. |
| Pros and cons to the process, timeline one of the biggest negatives which impacted all other parts of engagement in the process. |
| We have offered comments on draft evidence reports and during open input periods. |
| Willingness of organizers and the panel to include patient views. |
| The time frame was compressed, more time would have been good. I'd actually say the experience was not negative or positive but more neutral. |

| |
|---|
| We've participated in a number of ICER reviews with varying outcomes/experiences, but continue to be concerned that ICER does not, in most cases, adequately take into account the individual needs of patients/providers/caregivers. |
| We have submitted comments to ICER on their value assessment framework broadly and on certain drugs. |
| We have had two ICER reviews of CF therapies. This was a drain on organizational resources. We are opposed to the use of Quality Adjusted Life Year (QALY) methodologies and would rather focus our efforts engaging on education decision makers about QALY and encouraging bans. |
| Any time we have patient input it is valuable. |
| I do not find they care about the patient perspective. Focus is on payer perspective. |
| U.S. Pain Foundation worked in part with other migraine and headache disease organizations for two ICER reviews of migraine medications. It was challenging, frustrating, and stressful with a mixed final outcome. I learned a lot from witnessing the process. That said, I really learned more from LADA's involvement in the lupus nephritis ICER review. I feel patients need to understand ICER, and become involved. And by working with ICER in a more productive way, I think end results could be more positive overall. |
| We participate on the organizational level, not within individual reviews. This has been positive in the sense that there are more opportunities for patients/patient orgs to contribute. However, I recognize frustrations about actual impact of patient engagement on V/HTA and continue to work with HTAs/VAs in the US and abroad. |

# Q6B What are reason(s) that your organization has not participated in a HEA? (mark all that apply) (HEA Non-Participating Groups only)

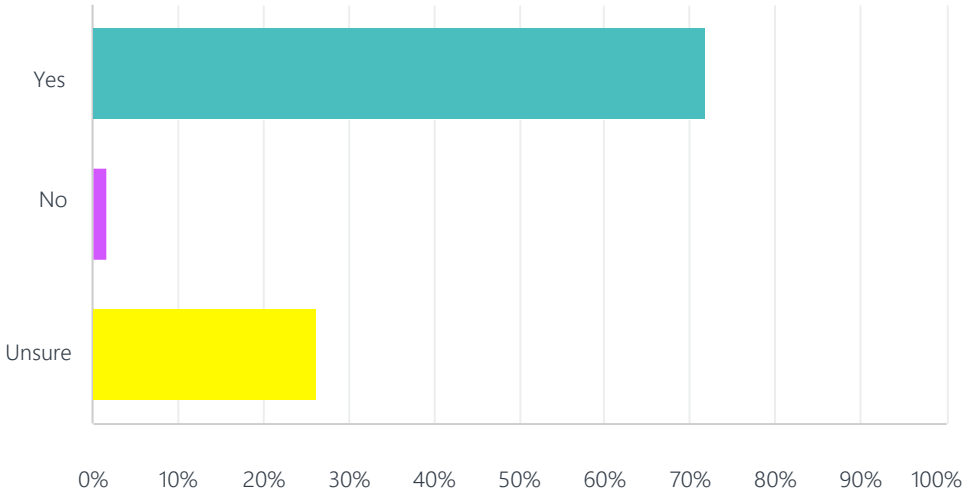



| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Lack of experience/expertise | 25.49% | 13 |
| Limited time | 23.53% | 12 |
| Limited staff | 35.29% | 18 |
| Overwhelmed by the process | 5.88% | 3 |
| Limited financial resources | 23.53% | 12 |
| Limited citable research data exists for your disease/condition | 7.84% | 4 |
| Not invited to participate | 68.63% | 35 |
| Not aware of them | 37.25% | 19 |

| OTHER (PLEASE SPECIFY) |
|---|
| We are an umbrella organization. |
| Our 1 approved drug was approved 20+ years ago, no new drugs since. |
| Not area of focus at the current time for any of our partners. |
| Drugs not reviewed. |
| This hasn't been an issue for us yet. |
| I think orgs may not participate for a combination of all of these. |
| As a convening organization, we support our disease-specific patient groups in their participation but have written in generalities about the importance of patient input and strongly discourage the use of the QALY in ICER work. |

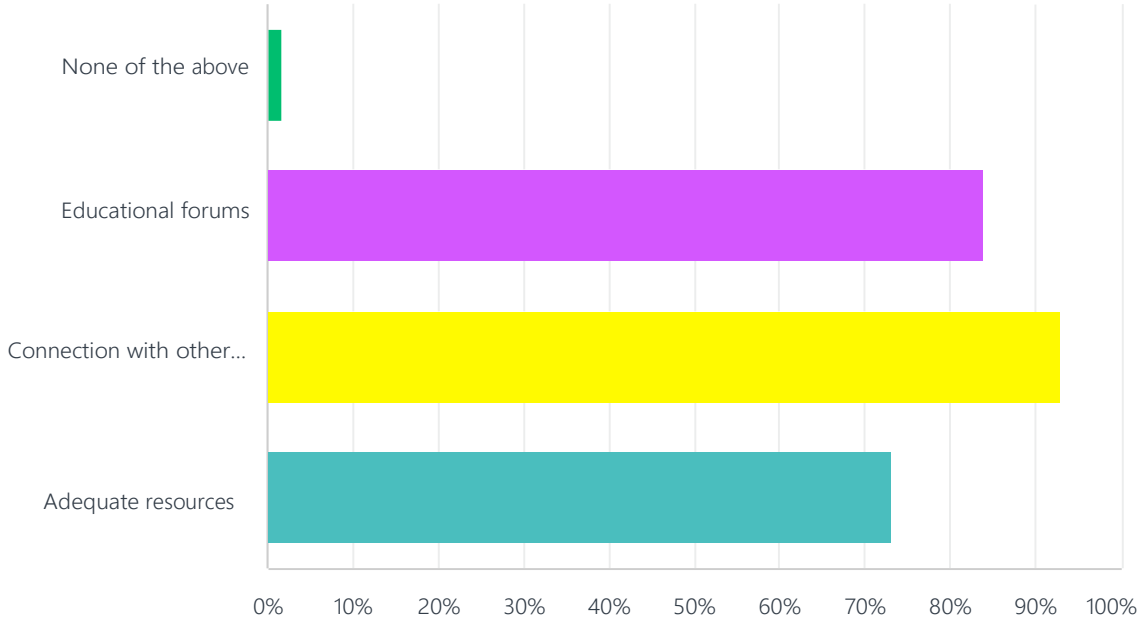
# Q7B Would you engage if you understood how to effectively participate in the process through educational forums, had adequate resources, and were able to connect with other organizations who had participated in HEAs? (HEA Non-Participating Groups only)

Answered: 55 Skipped: 0




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| Yes | 71.93% | 40 |
| No | 1.75% | 1 |
| Unsure | 26.32% | 14 |
| TOTAL | | 55 |

# Q8B Please select which resources you think would be most effective to help you participate: (select all that apply) (HEA Non-Participating Groups only)




| RESPONSE CHOICES | PERCENTAGE OF RESPONDENTS | # |
|---|---|---|
| None of the above | 1.79% | 1 |
| Educational forums | 83.93% | 47 |
| Connection with other advocacy organizations who have participated | 92.86% | 52 |
| Adequate resources | 73.21% | 41 |

| OTHER (PLEASE SPECIFY) |
|---|
| We are exclusively a policy and advocacy convening organization, so this does not apply |
| Gaining an understanding of whether this process will help us deliver a safe & effective treatment to our community. |
| Consultant assistance |
| Thank you for your leadership on this. |