

Furthering Patient Engagement in Health Economics and Outcomes Research: Exploratory Research by ISPOR and the National Health Council

Brian Ung, PharmD, RPh, MS; Zeba Khan, PhD, RPH, MS, Celgene Corporation, NJ, USA; Richard Willke, PhD, ISPOR, Lawrenceville, NJ, USA; Eleanor M. Perfetto, PhD, MS, National Health Council, Washington, DC, USA; Laura Pizzi, PharmD, RPh, MPH, Rutgers University, Piscataway, NJ, USA

ISPOR and the National Health Council have an opportunity to leverage their organizations' strengths and member expertise to aid patient advocacy groups and help them understand how their patients and members can be best represented in health technology assessments or value frameworks.

Patient advocacy groups (PAGs) or voluntary health groups have proven to be valuable research partners in helping identify patients with certain genetic or phenotypic markers; educating patients, providers and other audiences; and aiding patients with travel to and from study sites.¹ Health economics and outcomes research (HEOR) is an area in which patient engagement is increasing.^{2,3} Patient advocacy groups, as strong advocates for patients, may see value in conducting HEOR activities but may lack the expertise and/or resources to complete such a study. For example, a patient group may have existing data but lack the resources or expertise to extract meaningful and impactful insights that could meet the needs of its members.⁴

HEOR professionals may also benefit from working with patient advocacy groups. Individuals early in their careers may be able to obtain meaningful claims data or patient survey data projects or modeling experience through their interactions with patient advocacy groups. Fostering meaningful patient engagement behavior and gaining experience in this area could aid in the development of best patient-centered research practices.

The National Health Council (NHC) and ISPOR have built upon the established work of the Patient-Centered Outcomes Research Institute (PCORI) to increase the ability of patient advocacy groups to be an active participant in HEOR activities and healthcare discussions. ISPOR and NHC have an opportunity to leverage their organizations' strengths and member expertise to aid patient advocacy groups and help them understand how their patients and members can be best represented in health technology assessments or value frameworks.

Prior to ISPOR and NHC developing a mechanism to connect patient advocacy groups to HEOR professionals, more had to be learned about the characteristics,

research capabilities, and unmet needs of patient advocacy groups. Patient advocacy groups vary in size, disease area of focus, and research capabilities and may be composed of individuals with a variety of backgrounds. Therefore, an environmental assessment was necessary for optimal matching between patient advocacy groups and HEOR professionals. More information was also needed from the patient advocacy group and the HEOR professional perspective to better understand how the potential partnership of 2 groups can lead to mutually beneficial outcomes. This study sought to investigate the research knowledge, perceptions, and experiences of patient advocacy groups as they relate to HEOR activities. Additionally, this study adds to the literature by providing insight to the level of engagement and interest HEOR professionals have in collaborating with patient advocacy groups on future projects.

PATIENT ADVOCACY GROUP QUALITATIVE SURVEY

A semistructured interview guide was constructed to further understand the needs of patient advocacy groups. The interview guide had the following objectives: (1) understand the HEOR needs of patient advocacy groups, (2) understand the level of knowledge of key HEOR terms, (3) explore the expectations and perceptions of HEOR, (4) identify the level of resources available to conduct HEOR, and (5) document past experiences with HEOR.

A 6-phase thematic approach to analysis was utilized:

1. Getting familiar with the data
2. Generating initial codes
3. Interpreting and sorting codes into themes
4. Reviewing themes for coherent patterns
5. Defining and naming the themes
6. Producing the report⁵ >

Thirteen participants representing 11 different patient advocacy organizations, identified through the NHC network, took part in 11 separate interviews. patient advocacy groups had little to no staff specifically devoted to HEOR, and 4 interviewees stated that they were the sole person who participated in HEOR activity. Two themes were identified from the patient advocacy group interviews—increasing understanding of patients and value assessment and access to medication and healthcare.

INCREASING UNDERSTANDING OF PATIENTS AND VALUE ASSESSMENT

Several groups reported a desire to increase their knowledge of the costs incurred by patients throughout their lives. Examples of the different types of costs that patient advocacy groups sought to understand better included out-of-pocket costs, indirect medical costs, and overall societal costs of a disease. When asked to define outcomes research, patient advocacy groups tied their definition back to how a patient feels after receiving a treatment and how this impact compares to other treatment approaches the patient could have taken. Examples included symptom reduction, improvement in disease status, avoidance of hospitalizations, and economic outcomes.

with providing the patient and disease expert perspective in the development of their drug evaluations models. patient advocacy groups expressed that current value assessment tools, such as those utilized by ICER, did not entirely represent the priorities of their patient communities and that they wanted to increase the patient voice in healthcare discussions. Patient advocacy groups did not always feel that the ICER model was able to capture all areas of the disease accurately and therefore sought to shift the value assessment conversation to be more patient-centric. They wanted tools to quantify the total impact of a disease on a patient that extended beyond out-of-pocket costs and assessed their ability to work and maintain relationships with family and friends. Patient advocacy groups stated that the lack of internal resources (ie, funding, staff) limited their ability to proactively engage in value assessment and HEOR activities. Several groups reported having pieces of relevant information needed for the value discussion but lacked internal expertise to communicate the viewpoint of a patient advocacy group. Partnering with a HEOR professional was cited as an approach to fill this gap and be a stronger player in future value discussions.

with physicians to write statements to combat utilization management techniques (ie, prior authorization, step therapy) used by payers or to help form their organizational positions on health policies. In addition to research, clinicians are also brought in on an ad hoc basis for several purposes, including identifying clinically significant outcomes, developing patient registries, advising on advocacy positions, or leading epidemiological studies. Healthcare access issues stemming from ensuring preventative services related to the conditions they represented remained within current insurance requirements was also another cited obstacle.

Multiple patient advocacy groups stated how such conversations reach a point where actual data are needed to support anecdotal evidence from the patient advocacy group community. Having a HEOR professional assist in conducting further analysis on patient subgroups was cited as being beneficial in patient advocacy groups having more nuanced discussions with payers. Groups also cited not having the data or information necessary when engaging with ICER throughout the review process. Increasingly, patient advocacy groups are seeing the need to have such data earlier in the drug development process to avoid barriers to access to medication shortly after regulatory approval.

The overwhelmingly positive interest level in health economics and outcomes research professionals expressing a desire to work with patient advocacy groups highlights that future partnerships are possible. The variety of volunteers from different employment sectors and varying years of experience indicate that diverse skill sets are available to meet the needs of a patient advocacy group.

All patient advocacy groups strongly believed that the definition of value depended on the perspective (ie, patient, provider, insurer) and should be extended beyond efficacy, safety, and cost. Nearly all groups sought to understand value from the patient perspective. They believed that a partnership with a HEOR professional would help them understand how to quantify value in ways that demonstrate that the patient voice is heard.

Patient advocacy groups have also been contacted by the Institute for Clinical and Economic Review (ICER) to assist

ACCESS TO MEDICATION AND HEALTHCARE

Patient advocacy groups expressed the need to demonstrate the value of treatments from the patient perspective in their conversations with payers when discussing access. Several groups believed that access to medication decisions were being made without proper representation of the patient opinion and noted that payers were making decisions about access to medications based on outcomes they viewed as not being the most clinically relevant or appropriate. Patient advocacy groups discussed partnering

HEOR PROFESSIONAL QUANTITATIVE SURVEY

An 8-item survey was developed to understand the level and type of interest and engagement HEOR professionals have in working with patient advocacy groups on a volunteer basis. The survey was created to gather information about researcher's area of expertise, employment, years of experience, previous experience working with patient advocacy groups, level of interest volunteering with a patient advocacy group, size of project they would want to be involved with, readiness to volunteer, and specific patient advocacy groups or diseases of interest.

The HEOR professional survey was sent out to a total of 4328 ISPOR members in late 2017; members surveyed included all those in an ISPOR Special Interest Group, the Student Network, or the

Faculty Adviser Council. A total of 235 participants completed the HEOR professional survey, leading to a 5.42% response rate. Respondents were most often employed within life sciences, academics, and consulting organizations. Students also made up a fifth of the participants. The majority (90.6%) of respondents reported interest in working with a patient advocacy group, with over half stating they were very interested.

As this initiative moves forward, ISPOR and NHC are seeking both HEOR scientists and patient advocacy groups who are interested in engaging in small-scale pilot projects.

Participants were open to a variety of potential patient advocacy group collaborations as all project types had over a 20% response and the majority were ready to begin work within 6 months. Oncology was the most popular disease state of interest, but most participants did not state that they had a specific interest in a patient advocacy group or disease. There was a statistically significant relationship between years of experience, as those who were earlier in their careers reported being more likely to express interest in working with a patient advocacy group ($P < 0.008$).

PATIENT-RESEARCHER PARTNERSHIPS—REALIZING THE POTENTIAL

The potential for a HEOR professional and patient advocacy group partnership would allow patient advocacy groups to better understand their patients and their financial, clinical, or emotional burdens as well as value assessment methodology and HEOR. The collaboration will help patient advocacy groups circumvent their lack of internal expertise and resources and will allow them to be more well informed and generate the necessary data when having policy, advocacy, and healthcare access discussions on behalf of their patients. The overwhelmingly positive interest level among HEOR professional survey respondents in expressing a desire to work with patient advocacy groups highlights that future partnerships are possible. The variety of volunteers from different

employment sectors and varying years of experience indicate that diverse skill sets are available to meet the needs of a patient advocacy group. Replicating this work among patient advocacy groups based in other countries would aid the relationship between both groups internationally. Further research on the exact patient advocacy group needs will help determine feasible projects that can be accomplished within the workload and timeframe expressed by HEOR professionals. Communication of project scope and goals of both sides is paramount prior to entering into a patient advocacy group and HEOR professional collaboration. Additional communication and vetting of the HEOR professional will allow a more accurate determination of individuals with the right balance of time, experience, and ability to successfully collaborate with a patient advocacy group.

WANT TO GET INVOLVED?

HEOR professional and patient advocacy group collaboration has the potential to be mutually beneficial for both parties. As patient engagement and the patient voice continue to be a larger part of the healthcare value discussion, collaborations between both groups can help patient advocacy groups be a more effective contributor in the assessment of health technologies. As this initiative moves forward, ISPOR and NHC are seeking both HEOR scientists and patient advocacy groups who are interested in engaging in small-scale pilot projects. •

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ADDITIONAL INFORMATION

The preceding article is based on a presentation given at ISPOR 2018. To view the presentation, go to https://www.ispor.org/docs/default-source/presentations/1421.pdf?sfvrsn=8dba379c_1.

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