Involvement of Patients and Medical Professionals in the Assessment of Relative Effectiveness: A Need for Closer Cooperation

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ABSTRACT

Objectives: Involvement of patients and medical professionals in assessment of relative effectiveness (relative effectiveness assessment) contributes to an efficient and effective health technology assessment (HTA) process and supports acceptance and implementation of the outcome. This study aimed to analyze stakeholder involvement in assessing relative effectiveness and how the parties involved value this collaboration.

Methods: This is a document analysis of all drug assessments completed in 2019 (20) by the public HTA agency of The Netherlands, enriched with semistructured interviews with employees of the HTA agency (18) and representatives of patient (5) and medical (11) associations involved in these assessments. Data were analyzed, coded, and categorized.

Results: In almost half of the assessments, there was no coordination with the medical associations at the start of the relative effectiveness assessment and no patient associations involved in this phase. During the assessment procedure, patient and medical associations were always asked to comment on the draft report. Nevertheless, the strict 5-day deadline that the HTA agency uses as a response period often hampered a proper response and involvement. According to interviewees of the HTA agency, this leads to a great diversity in the substantive quality of their input. Patient and medical associations indicated that the HTA agency relies too much on “paper knowledge,” which leads to a (perceived) lack of alignment with clinical practice.

Conclusions: The limited involvement results in a lack of coordination and mutual trust. Optimizing involvement of patients and medical professionals in HTA practice requires effort from all parties involved. Procedural adjustments and better coordination, especially at the start of the assessment, would probably improve cooperation.

Keywords: communication, health policy, health technology assessment, information sharing, patient involvement, professional involvement, relative effectiveness assessment, stakeholders.

Introduction

New and expensive drug therapies continuously enter the market, putting pressure on the accessibility and affordability of care. Health technology assessment (HTA) is an essential step in promoting an equitable, efficient, and high-quality health system, for after a new drug has been approved, an important question for patients, healthcare professionals, and payers is how much benefit it provides compared with available alternatives.1 Assessment of this relative effectiveness assessment (REA) is a cornerstone of HTA in many countries.2,3 The result of this analysis is one of the most important elements in reimbursement decisions.4,5

Besides scientific evidence, many HTA organizations involve the input from patients and medical experts in their work.7,8 HTA organizations differ in the degree of stakeholder involvement: ranging from informing stakeholders about process and outcome to active participation in the entire HTA process.9-11 Consequently, the impact of this involvement varies.12,13

Much of the literature on stakeholder involvement in HTA focuses on patients, emphasizing the importance of their participation.10,13,14,15 describing their role and position in the HTA process,10,13,15,16 or exploring their input and impact.12,13,17 The small number of articles on the role of professionals in HTA is mainly descriptive in nature and compares the role of professionals in the different national HTA organizations7,8,14 or focuses on the role of professionals in relation to real-world evidence.18-20 We aim to contribute to this knowledge by analyzing and evaluating the interaction of patients and medical professionals with the public HTA agency in The Netherlands (Zorginstituut Nederland [ZIN], the Dutch National Health Care Institute) in performing REAs of drugs. By combining factual information from several HTA assessments with a retrospective qualitative evaluation of the process, a rich picture is obtained of the interaction between the parties involved.

ZIN advises the Minister of Health, Welfare and Sports (MoH) on the content of the benefits package, based on 4 formal criteria:
Assessment of relative effectiveness is the first step and a positive conclusion is a prerequisite for taking the next step: assessment of budget impact and CE. REA, budget impact, and CE are submitted for an appraisal by the Insured Packages Advisory Committee. In a public session, the Insured Packages Advisory Committee formulates an advice to the board of the National Health Care Institute. The board is legally responsible for an advice to the MoH. If a REA is negative, no financial analysis is made, the assessment procedure stops, and a negative advice to the MoH follows. Because of the important role of the REA in the formation of the advice (relative effectiveness is a knockout criterion), we focused on the REA in this study.

When assessing relative effectiveness, knowledge and experience of patients and professionals are taken into account. As described in the policy documents, ZIN aims to obtain this information in 2 steps in the assessment process (Fig. 2). At the start of the assessment, to formulate relevant starting questions based on the Population, Intervention, Comparison, and Outcome (PICO) framework, information is collected from (international) guidelines, and in addition, patient and medical professional associations can be consulted about matters such as which outcome measures should be chosen. Later in the process, the draft version of the assessment is submitted to stakeholders for substantive comment.

Based on our aim of contributing to the knowledge about the role of patients and medical professionals in HTA, we focused on the following 2 research questions:

1. What role do patients and medical professionals have in the realization of a REA?
2. How is this cooperation experienced and valued by the involved stakeholders?

In the next section, we will briefly explain the quantitative and qualitative methodological aspects of our study.

Methods

This descriptive study is based on a document analysis of assessments completed by ZIN and on interviews with employees of ZIN and with the representatives of the patient and medical associations involved in these assessments.

Sample

A period of 1 year (January 1, 2019, to December 31, 2019) was chosen to obtain a substantially varied and representative number of assessments. Analysis was limited to assessments of new drug therapies; other new medical technologies were excluded. These criteria resulted in a sample of 20 completed assessments.

Data

Data were retrieved in 2 phases. The first phase consisted of a semiquantitative document analysis. The data were obtained via ZIN's digital archive and from the institute’s website. ZIN’s website contains the final reports on the REAs performed and the minutes of the meetings of the Scientific Advisory Board (SAB). In 2019, a total of 35 assessments on relative effectiveness were conducted: 15 of these REAs did not involve patient and medical associations because they were marginal assessments. ZIN can decide to conduct a marginal assessment without input from stakeholders if, for example, an analog drug has already been assessed. Because the focus of the study is on the interaction between ZIN and the associations, only full assessments were selected involving patient and medical associations (n = 20) (Fig. 3).

The digital archive contains additional documents underlying the production process of the assessments (approximately 40-90 documents per case). All documents were manually searched for interactions with all stakeholders during the assessment. This search was performed by the first author, after which checking and completion were done by the second author. Data on interactions with patient and medical associations were extracted for further analysis.

The data retrieved were analyzed and categorized based on the time of input into the process (eg, during the scoping or in response to the first draft) and on content (eg, input about the outcome measure, treatment algorithm, or minimal important difference). A systematic comparison of the contributions from patient and medical associations during the production process of the assessments with the final reports revealed whether and how ZIN had used the input and what the impact of their contributions had been.

The results were included in a spreadsheet based on elements of the Hutton framework. The descriptive Hutton framework provides a structure to analyze and describe HTA systems, distinguishing between policy implementation and technology decision levels. The policy implementation level describes how a reimbursement system relates to the broader political system; the technology decision level analyzes the process of an individual reimbursement request and its 3 stages (assessment of evidence, decision making, outputs and implementation). Although the Hutton framework was developed to compare HTA systems and our research focuses on an analysis of individual HTA procedures within a single HTA system, elements of the technology decision level were valuable as a starting point for our research. In the analysis of the collaboration between the HTA agency with patient and medical associations, we have included the following elements: consultation and involvement in the assessment (scoping) phase (“Were the associations involved, and if so, how and on what points?”), involvement in and input to the conclusion of the REA, and involvement in agreements about possible implementation (see Appendix 1 in Supplemental Materials found at https://doi.org/10.1016/j.jval.2022.03.021).

The second phase of the data collection consisted of semi-structured interviews with all assessors and patient and medical associations involved in the 20 assessments in our sample. A total of 34 interviews were conducted with employees and external advisors of ZIN (assessors, secretaries, and the members of the SAB, n = 18, involved in all 20 assessments), representatives of patient associations (n = 5, involved in 8 assessments, of which 3 were negative outcomes, meaning that ZIN advised against the therapy’s uptake in the reimbursement package), and medical experts as representative of their association (n = 11, involved in 11 assessments, of which 4 were negative outcomes). In the selection of respondents from the patient and medical associations, we considered an equal reflection of oncological and nononcological drugs and of HTA procedures with positive and negative reimbursement outcomes. The number of interviewees from the patient associations is somewhat smaller because not all indications that were assessed in the HTA procedures have patient associations, and because only 1 patient association is involved in the assessment of all oncological agents. All persons approached for an interview accepted. Data saturation was discussed among authors and provided grounds for determining the final number of interviews conducted.

The purpose of the interviews was to gain an in-depth picture of ZIN’s collaboration with patient and medical associations, with the selected cases serving as examples of this collaboration.
Semistructured interviews were conducted, based on a topic list (see Appendix 3 in Supplemental Materials found at https://doi.org/10.1016/j.jval.2022.03.021). The topic list was formed based on elements from the Hutton framework and on the results of the document analysis. The following topics were covered: involvement of patients and medical professionals (ie, role, Figure 2. Assessment of relative effectiveness, formal process, and input from stakeholders (emphasized in figure).

ZIN indicates Zorginstituut Nederland.

Figure 1. Sequential steps in the assessment and appraisal of new drug therapies by the Dutch National Health Care Institute (ZIN), assessment of relative effectiveness emphasized in figure.

MAH indicates market authorization holder.
procedural position, impact), role of scientific and experiential evidence (use of the GRADE-method in judging the applicability and strength of evidence in HTA, internal and external validity, guidelines), ZIN's working method (procedural, methodological), and the perceived mutual cooperation.

The interviews took place between March 2020 and February 2021 and lasted 45 to 90 minutes. Most interviews were conducted by telephone or by video calling, because of social restrictions because of COVID-19. Conversations were recorded, transcribed verbatim, and coded. During the study, the required ethical guidelines were followed: all interviewees received extensive information in advance and signed an informed consent (Appendix 2 in Supplemental Materials found at https://doi.org/10.1016/j.jval.2022.03.021), data were anonymized after verbatim processing, and participants in the study could withdraw at any time. In our Results section, all quotes were anonymized, translated from Dutch, and approved by the relevant interviewees.

All interviews were systematically coded by the first author, and in addition, all members of the research team each read a selection of transcripts and checked the codes. Halfway through the series of interviews, the research team discussed the preliminary results of the coding. Based on inductive analysis, 3 broad domains emerged: cooperation characteristics, the assessment procedure, and handling uncertainties. These domains recurred in all interviews; therefore, the research team has chosen to further elaborate the analysis based on this classification. In iterative coding sessions, these 3 domains were subdivided and categorized into more specific themes. All interviews were elaborated based on these domains and themes. Validity of the analysis was addressed by comparing consistency of domains across different interviews, by discussion within the research team, and by presenting the results to the interviewees, both individually and during a group meeting.

Results

Based on the data from the semiquantitative document analysis, an extensive overview was constructed, showing the details of the interaction between ZIN and patient and medical associations (see Appendix 1 in Supplemental Materials found at https://doi.org/10.1016/j.jval.2022.03.021). A summary of this is presented in Table 1 (see below). All of the analyzed assessments were created in the same step-by-step manner, corresponding to the description as in Figure 1. The elaboration of the different steps and the degree of involvement of the patient and medical associations did differ, as described in this section.

Given that the results of the interviews strengthen the substantiation and interpretation of the semiquantitative analysis, both sources will be combined in presenting the results. In reporting our findings, we follow the aforementioned division into 3 broad domains: cooperation, procedure, and handling uncertainties. The text below first describes the results of the document analysis for each domain and then the results of the interviews.

Cooperation

"Input from patients and medical professionals is important in the assessment procedure," so ZIN states in policy documents.20-22 This input is formally obtained in different ways: consultation of professionals' guidelines, participation in meetings, or through written communication with stakeholders. How this is put into practice differs greatly from one assessment to another (see Table 1).

The document analysis shows that medical professionals' guidelines are an important source of information to the assessors of ZIN: in 17 (of 20) cases these were used, mainly to extract information about treatment algorithm, outcome measures, and

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**Figure 3. Selection of studied REAs and respondents.**

- 35 REAs in 2019
- 20 assessments with involvement of patient and medical associations
  - Characteristics:
    - 7 out 20 oncological drug therapies
    - Recommendations based on REA: 12 positive, 3 partially positive, 6 negative
- Interviews:
  - Representatives patient associations: 5 (involved in 8 REAs, of which 3 negative outcome)
  - Representatives medical associations: 11 (involved in 11 REAs, of which 4 negative outcome)
  - Employees of ZIN: 18 (involved in all 20 REAs)
- 15 marginal assessments, no involvement of patient and medical associations, excluded

REA indicates relative effectiveness assessment; ZIN, Zorginstituut Nederland.
minimal important clinical difference. In 3 assessments, no national guidelines were available; in 2 of these cases, a local (hospital) guideline was used instead.

In the early phase of the assessment, in addition to information from guidelines, the assessors from ZIN can retrieve information by approaching medical associations. The document analysis showed that this occurred in slightly more than half of our cases (n = 12), especially to collect missing information if guidelines left questions unanswered or if, for example, there were doubts about the applicability of the available scientific evidence in relation to clinical practice. Besides medical associations and also patient associations can be involved in the early assessment phase, for example, to give input about appropriate outcome measures, but this hardly occurred (n = 3).

When the first draft of the assessment is completed and discussed within the SAB, patient and medical associations are asked for their response by default. In our sample of 20 assessment, medical associations were invited to respond in all cases. Patient associations were invited in 17 cases; in the other 3 cases, there were no patient associations for the specific disease. In all 20 assessments, the medical associations invited to do so responded; of the patient associations invited in the 17 assessments, no response came from 1 patient group. The document analysis showed that the content of these responses varied widely. Although some associations responded in great detail, others responded in 1 or 2 sentences unrelated to the issues at hand. The impact of the associations’ contributions was limited: only in 2 assessments were the final conclusions of the assessment altered. In a few reports, some minor adjustments were included, without having an effect on the final conclusions.

Besides the assessment of the new drug therapy, ZIN sometimes makes additional agreements with the associations of medical professionals on appropriate use or monitoring. Nevertheless, this was seldom done in 2019. In 2 assessments, ZIN made agreements with the medical association about monitoring; in another one, conditions for appropriate use were agreed upon; in a fourth assessment, both monitoring and conditions for appropriate use were established. In all 4 of these assessments, the medical associations were involved in these agreements; patient associations were involved in 1. In 3 assessments with a negative outcome, the medical associations proposed conditions for appropriate use in an attempt to gain limited access to the treatment; these proposals were rejected by ZIN.

The interviews revealed that all stakeholders recognize the legitimacy and importance of everyone’s role and contributions in the assessment. Nevertheless, there were also some critical comments. Most representatives of patient and medical associations experienced ZIN as a “black box.” This leads to feelings of mistrust on their behalf. Several factors fuel this: unfamiliarity with the role of ZIN and with the assessment process; the limited possibilities for oral contact, discussion, and consultation; and the idea that the MoH exerts influence on ZIN, making financial considerations leading.

“I am convinced that they are sometimes misusing evidence-based medicine and using false arguments to avoid expensive treatments. And I believe they have been ordered to do this by the Ministry.”—Medical professional number 3.

From the interviews, not only a critical attitude of the patient and medical associations toward ZIN emerged; this attitude also exists the other way around. The first point of criticism is very practical: it is often not clear who ZIN should contact or a request for cooperation is only responded to after insistence. Second, half of the interviewed assessors indicated that the input of the patient and medical associations may not be objective because of their personal involvement with the disease, with patients, or in sponsored scientific research. Although this involvement is exactly the reason why these experts are invited to give input, this means that input from patient and medical associations is sometimes appreciated with some reticence.

“A doctor has other views besides the purely technical and scientific ones. And he [sic] must defend the personal interest of that 1 patient in the treatment room, too.”—Institute’s employee number 6.

Interestingly, ZIN’s critique on patient and medical associations is shared by half of the interviewed patients and medical professionals. They confirm the potential bias of their input, but nonetheless consider their personal and clinical experiences to be of value in addition to the scientific data. In the assessments, these experiences should be taken more into account, they believe.
“A patient is better able to judge what a relevant outcome measure is, or to weigh the risks of a treatment. And gives colour to a clinical picture so that ZIN understands it better.”—Patient representative number 3.

**Procedure**

The assessments done by ZIN follow a transparent and consistent procedure.20-22 Formal regulations influence the cooperation with patient and medical associations: communication back and forth is preferably in writing to secure traceability, and contact is not directly with the assessors but with the coordinating secretary. Furthermore, the assessments are done under time pressure: the total turnaround time for the assessment is set at a maximum of 90/120 days, and patient and medical associations are given 5 working days to respond to the draft report. All interviewees—patients, medical professionals, and ZIN’s employees—indicate that these rules regularly stand in the way of efficient and good cooperation.

The time restrictions of the assessment procedure are the main limits. More involvement at the start of the process by means of scoping is seen as desirable by most interviewees. It would lead, according to the interviewees, to a greater understanding of the disease and better alignment between clinical practice and the starting (PICO) questions underlying the assessment. The reason that scoping is often done without or with marginal interaction with stakeholders is due to a lack of time, according to the assessors. Nevertheless, better coordination between stakeholders in the beginning of the process, the medical associations argue, would enhance efficiency later in the assessment.

A second moment when time pressure stands in the way of efficient cooperation is when a response to the draft assessment is requested. Patient and medical associations are requested to respond to this comprehensive draft report within 5 working days, often without prior notice to prepare. This strict 5-day deadline often hampers a proper response and involvement. According to interviewees of the HTA agency, this leads to delayed responses and a great diversity in the substantive quality of their input. An interviewed medical professional confirms this bottleneck:

“The answer must be given within a short period of time, which means that there is a degree of arbitrariness in how correct that advice is. Then things really go wrong, the right arguments are not formulated and this is taken into account in the final report. I have experienced this a few times.”—Medical professional number 4

### Handling Uncertainties

The methodology underlying ZIN’s assessments was often discussed during the interviews, the central question being “how do involved stakeholders manage the uncertainties in the assessment?” Various types of uncertainty emerged from the interviews and the document analysis, as categorized in Table 2.

All parties involved recognize that every assessment is accompanied by uncertainty. The impact and nature of these uncertainties is experienced in different ways by ZIN, patients, and medical professionals. Institute staff and medical associations mainly cite uncertainties regarding the procedure and the use and interpretation of scientific data, such as uncertainties regarding internal and external validity.

Although ZIN’s assessors recognize these uncertainties, at the same time they all emphasize that the reviews are based primarily on factual and objective information. ZIN, they indicate, can work objectively for various reasons. First is because of the neutral position of ZIN and the focus on the collective, unaffected by individual interests. The second reason is that the assessments are based as much as possible on evidence based medicine (EBM) and GRADE methodology, which is seen by the assessors as the state-of-the-art method to arrive at a reliable and objective result. Finally, the internal procedures and checks and balances guarantee careful and objective decision making.

“You have to base yourself on the evidence, because your gut feeling often tells you that those graphs do look nice in those newly published articles. And you will also talk to people internally about such a graph and say: ‘Is this right, is it a plateau? And when do you call it a plateau?’ You just need to carefully follow all your steps in the procedure to come to a conclusion.”—Institute’s employee number 2.

Patient and medical associations appreciate the role of EBM in the assessments and recognize that ZIN can arrive at a more objective judgment. Nevertheless, according to the interviewees, ZIN mainly bases its assessments on “paper knowledge,” whereas knowledge about the impact and clinical practice of a disease that is necessary to correctly interpret and apply EBM is lacking. Moreover, according to the associations, ZIN often sets the bar too high by demanding too high a level of evidence.

“As long as the institute sticks to randomized controlled trials with hard outcome measures as a prerequisite for reimbursement, we don’t stand a chance. It is a very rare disease so a worldwide trial starting at young age and with a long follow-up time would

<table>
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<tr>
<th>Types of uncertainty in relation to relative effectiveness assessment</th>
<th>Mentioned by</th>
<th>Examples</th>
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<tbody>
<tr>
<td>External validity of scientific data</td>
<td>Institute, medical associations</td>
<td>Disease or treatment regimen heterogeneous, insufficient connection with clinical practice, multiple treatments not compared with each other, lack of real-world data</td>
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<tr>
<td>Internal validity of scientific data</td>
<td>Institute, medical associations</td>
<td>Too small patient groups, no randomization, intercurrent treatments</td>
</tr>
<tr>
<td>Patient group vs the individual</td>
<td>Patient associations</td>
<td>Effectiveness for the collective does not necessarily equate to value for the individual</td>
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<tr>
<td>Procedural</td>
<td>Institute, medical associations</td>
<td>Choice of outcome measure, method of analysis (effectiveness, ITT/mITT)</td>
</tr>
<tr>
<td>Interests of patients and medical professionals</td>
<td>Institute, patient, and medical associations</td>
<td>Emotional involvement of/with patients or involvement in research</td>
</tr>
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ITT indicates intention-to-treat; mITT, modified intention-to-treat.
be needed before you could start seeing results. Of course, that is not going to happen."—Medical professional number 8.

Besides the uncertainties related to the use and interpretation of scientific data, patient associations emphasize especially the lack of focus on the interest of individuals in the assessment processes. A drug, according to patient associations, may well be important to an individual while having a minor effect at the group level, and this is not sufficiently taken into account.

Both patient and physician associations envision the same solutions for dealing with their objections: patient and medical associations should be more involved at the beginning of an assessment, in the formulation of the starting questions (PICO), and in determining the essential features of the assessment (eg, the outcome measures and the standard treatment against which the new treatment is compared). A second possible solution, mentioned by both patients and professional representatives, is that assessments should be more often tailored and adjusted to the level of evidence that can reasonably be obtained. Although ZIN's policy documents state that assessments are based on “appropriate evidence” and that this can also be lower forms of EBM evidence, this is not perceived by the representative associations. Third, patient and medical associations argue in favor of paying more attention to subgroup analysis in assessments. Some treatments do not appear to be effective for the entire patient population, but they are effective for a subgroup. Agreements on appropriate use could be made to facilitate access for this subgroup.

Discussion

From previous research, it is known that involvement of patients and medical professionals in HTA can lead to a more supported process and better acceptance and implementation.\(^\text{10,14,15}\) The importance of involvement of patients and professionals is emphasized in ZIN's policy notes: involvement is aimed at least 2 moments of the assessment (during scoping at the beginning and later, when commenting on the draft is requested) and consists primarily of involving current medical guidelines and consulting patient and medical associations. Despite this policy, participation in practice seemed less extensive. Our study revealed 2 main bottlenecks. First is the limited involvement of the associations: in almost half of the assessments, there was no coordination with the medical associations at the start of the assessments, and patient associations were hardly involved at all in this scoping phase. Although medical guidelines have been consulted as a source of information in almost all assessments, this is insufficient in the opinion of the patient and medical associations. According to the interviewees, additional consultation with the associations will lead to a more optimal coordination on essential parts of the assessment, such as the outcome measures, the relevant patient group, and the standard treatment with which the new treatment is compared. Coordination at the start can prevent disagreements later in the assessment and leads to a better connection with clinical practice.\(^\text{10,15,25}\)

The second bottleneck we identified occurred later during the assessment, when ZIN requested comments on the draft. Although all relevant medical and patient associations were invited in all cases to comment and virtually all associations responded, qualitative analysis based on interviews revealed that the strict 5-day deadline that ZIN uses as a response period hampered a proper response and involvement. This is in line with previous international research\(^\text{8,17,26}\) and could explain the often-limited contributing relevance and impact of the associations' contributions on the outcome. Relatively small adjustments in the process might already improve the cooperation: for example, associations can be notified in advance that an assessment is imminent, and the time to respond to a draft could be extended. Although the latter can lead to a delay in the assessment (and thereby in patients' access), several interviewees indicated that this could be compensated for by working more closely together earlier in the process.

Finally, all parties involved indicate that available scientific evidence often provides insufficient guidance to reach a certain conclusion, which means that each assessment is accompanied by various uncertainties. Following on from previous studies, different types of uncertainties surrounding assessments were identified by interviewees. Nevertheless, the interviewees of ZIN believe that their neutral position and careful substantive and procedural approach enables them to assess these uncertainties objectively. The objections of the patient and medical associations against this are 2-fold: first, ZIN relies too much on paper knowledge, lacking a connection with clinical practice. Second, although ZIN's policy documents state that assessments are based on “appropriate evidence” and that this can also be lower forms of EBM evidence, this is not perceived by the representative associations and they believe that often too high a level of evidence is required. Therefore, the patient and medical associations argue for more involvement, especially at the start of the process, and a more tailored assessment in line with the specifics of a disease's reasonably available scientific evidence.

Strengths and Limitations

This study gives a unique and in-depth insight into stakeholder involvement in HTA in daily practice. Nevertheless, it is based on the analysis of a limited number of assessments (20). Nevertheless, triangulation of data through document analysis and interviews with relevant stakeholders and member-checking ensured the validity of research and resulted in a broad scope of the various dimensions of the interaction of patients and medical professionals with HTA. Another limitation concerns the possible influence of interviewed patients and professionals by the pharmaceutical industry. Although the authors have no evidence that the patient and medical associations involved in the study are sponsored, it cannot be excluded that interviewed representatives of the associations have a relationship with the pharmaceutical industry.

HTA is a complex activity in which new insights and innovative medicines lead to changes in HTA practices. Findings from 2019 may already be partially outdated. This necessitates constant research into these new developments. So far, little research has been done on the role of medical professionals in the HTA process. With this study, we aim to contribute to this knowledge. We focused on the assessment of the REA and did not include the appraisal phase in our study (whereby in The Netherlands patient organizations and medical associations are invited to give oral input to the appraisal committee). Follow-up research on the collaboration between HTA bodies and representatives of patient groups and medical professionals can explore several aspects, for example, by comparing the characteristics of involvement in HTA procedures in different countries. Analysis of the role of quality of life studies in the assessment would be another interesting approach for further research. Finally, it would be interesting to
identify how different institutional mechanisms can facilitate patient and practitioner input.

Conclusions

Involvement of patients and medical professionals in HTA can contribute to an efficient and effective HTA process and support acceptance and implementation of the outcome. Document analysis of the 2019 assessments by the HTA agency in The Netherlands and interviews with assessors, patients, and medical professional representatives show that involvement of patient and medical associations in the scoping phase was limited. During the assessment procedure, patient and medical associations were always asked to comment on the draft report. Nevertheless, the strict 5-day deadline that the HTA agency uses as a response period often hampered a proper response and involvement. According to interviewees of the HTA agency and medical professionals, this leads to a great diversity in the substantive quality of their input. Moreover, patient and medical associations indicated that the HTA agency relies too much on “paper knowledge,” which leads to a (perceived) lack of alignment with clinical practice. The limited involvement results in a lack of coordination and mutual trust.

Despite the bottlenecks in collaboration, all parties value each other’s input and the HTA agency is appreciated for its role in bringing together and weighing scientific insights with input from various stakeholders. Improvement of coordination between the HTA agency and patient and medical associations will demand effort from all sides. Procedural adjustments and better coordination, especially at the start of the assessment, would probably improve mutual cooperation.

Supplemental Material

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