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EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT

# Patient Input in Relative Effectiveness Assessments

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# Patient Input in Relative Effectiveness Assessments

Health Technology Assessments (HTA) are decision support tools that summarize information about medical, social, economic, organisational, ethical issues and patient perspectives in a transparent, systematic and rigorous manner (1). Relative Effectiveness Assessments (REAs, synonym: Rapid REAs) compare a specific health technology to one or more relevant alternative interventions, using a limited subset of domains from the HTA Core Model® to focus on a technology's clinical effect and safety. REAs are typically performed more quickly than an HTA analysis, which includes a larger set of Core Model® domains (2). There is international agreement about the importance of involving patients in HTA and decision processes (3). Some examples of international organizations with initiatives for patient involvement in HTA are: the Federal Joint Committee (G-BA) (4), the National Institute for Health and Care Excellence (NICE) (5), the French National Authority for Health (HAS) (6), the National Institute for Health Research (7), Health Information and Quality Authority (HIQA) (8), the Scottish Medicines Consortium (SMC) (9), the Spanish Network of HTA Agencies (10) and the Canadian Agency for Drugs and Technologies in Health (CADTH) (11). In addition, Health Technology Assessment international (HTAi) has an interest group for Patient and Citizen Involvement in HTA (12).

Patient input is important for the evaluation of and decision-making related to health technologies (13-15). There are several published examples of international experiences with (13; 14; 16) and approaches to involving patients in HTA (15-19).

Patient contributions to the HTA process can be incorporated directly via individual or group input, which does not constitute primary research, or indirectly by conducting a synthesis of qualitative primary research (13). This document focuses on direct input. Although the Task Group recognized the value of using a qualitative evidence synthesis (QES) to capture patient views, the Task Group chose not to examine this method for patient input more closely at this time.

## **Aims**

This document reports the development of recommendations for direct patient input in The European Network for Health Technology Assessment (EUnetHTA) Relative Effectiveness Assessments (REA) process within the Joint Action 3 (JA3 2016-2020). Our goals have been to:

1. *Reach a common understanding of patients' input goals and preferred ways to participate in REAs within JA3*
2. *Describe patients and caregivers*
3. *Agree on the time frame for patient input*
4. *Describe preferred methods for patient input*
5. *Define how to make patient input visible in the assessments*
6. *Agree on confidentiality issues, conflict of interest and reimbursement*
7. *Agree on evaluation of the patient input process*

## **Process**

The EUnetHTA Secretariat established a Task Group, led by the EUnetHTA Senior Scientific Officer, on Patients and Consumers and Healthcare Providers. The Task Group consisted of representatives from Work Package (WP) 1, WP2, WP4, WP5 and WP6 Lead Partners and Co-Lead Partners within the JA3. The kick-off for the Task Group was in September 2017. The objective of the Task Group was to support the development of a process for Patient, Consumer, and Healthcare Provider involvement within WP4 assessments and WP5 early dialogues. The Task Group had weekly or bi-weekly e-

meetings that included discussions of proposals and experiences with stakeholder involvement in the Joint and Collaborative Rapid Relative Effectiveness Assessments. Two face-to-face consulting meetings with stakeholders representing European patient and consumer organizations, as well as other organizations from the HTA network stakeholder pool also took place. The first meeting was held in Diemen, Netherlands on 08.03.2017 and the second meeting was in Brussels, Belgium on 26.01.2018. Minutes from these meetings are available at <https://www.eunetha.eu/stakeholders/patients/>.

In this document, we describe the process regarding direct patient input in REAs within JA3. The document is primarily intended for those who design and conduct EUnetHTA REAs although it may be informative for a wider audience of patients, health care providers, payers, and industry stakeholders. Engagement of patients in other EUnetHTA activities, e.g., Early Dialogue (ED) or engagement of health care providers (HCP) will be described in additional documents.

This recommendation document will be attached as a consented guidance document in the annex of all SOPs that describe processes related to patient input in REAs. Several SOPs include process steps related to patient involvement.

### **1. Goals for direct patient input in REA**

The Task Group agreed that the overall goal for direct patient input should be to improve the applicability of REAs. Within a realistic framework for REAs in JA3, we decided that the preferred method for patient participation is to collect patient input in the scoping phase.

#### ***Goals for obtaining direct patient input:***

- *to collect patient input regarding:*
  - o *their disease/condition and their unmet needs*
  - o *currently available treatments*
  - o *expectations with respect to new treatments (e.g. fewer side effects)*
  - o *identification of subgroups and possible effect modifiers*
  - o *quality of life issues*
  - o *target treatment population and risks of off-label use*
- *gather information about outcomes that are important and relevant from a patient's point of view*

Patient input can provide important insights into the disease and treatment process that can guide the assessment team's selection of relevant outcomes measures for an REA and potentially improve the REA's relevance, legitimacy and transparency.

### **2. Profile of patients and caregivers**

When discussing the profile of patients and caregivers, the Task Group agreed on both what patients and caregivers could bring to the REAs and who they might be.

The Task Group prefers gathering patient input from individual patients, either via patient organizations or via direct contact with patients. In some cases, however, there may be challenges associated with getting the patient's perspective directly, e.g. if the patient is a child or unable to communicate due to the condition. A caregivers' perspective may be useful in such cases.

Patients and caregivers may also act as patient or caregiver representatives, respectively, to represent the views of a particular group of patients, survivors or caregivers. It is important that they are clear about whom they represent, and the basis for their knowledge of patient perspectives. Below we highlight the benefits associated with including individual patients or caregivers.

#### **An individual patient**

An individual patient is a person with lived experiences of the health condition.

Patients can:

- *Bring a detailed knowledge of the experience of living with the health condition, including its burden on daily life the diagnostic process, and currently available treatment(s) (if any treatment is available).*
- *Share the experiences of other patients based on their personal interactions.*

### **An individual patient's caregiver**

An individual patient's caregiver is a person with experience acting in a primary care role for people living with a health condition. As with patients, caregivers may also act as representatives for a particular group of caregivers.

Caregivers can:

- *Bring second-hand knowledge about what it is like to live with the health condition, including its burden on daily life, the diagnostic process and currently available treatment(s).*
- *Caregivers are affected by the disease via their close relationship with the patient, and their perspective can be relevant regarding familial and social aspects*
- *Share first-hand knowledge about being a caregiver and/or guardian for individuals with the health condition*

### **3. Timeframe for patient input**

At this time, the Task Group decided that the focus should be on input during the scoping phase of the assessment process. The patient input can for example inform development of the PICO table and give the assessment team insights into patient experiences.

### **4. Preferred methods for patient input**

During the process of reaching consensus on and describing the preferred methods for patient input, the two WP4 Co-Lead partners, with input from WP5, recorded their experiences with patient input in assessments of other technologies and pharmaceuticals, respectively. Based on their experiences and discussions, the Task Group decided on the input methods and recruitment process described below. One method does not preclude the use of other methods.

The Task Group agreed that it is not mandatory for patients to have extensive knowledge about HTA or evidence-based medicine since the aim is to collect patient input in the scoping phase of the assessment. However, patients may receive some basic information before participation. WP2 has developed a patient information pamphlet that can be useful in this context.

The preferred methods for obtaining patients input are:

#### **Open call for patient input through patient organizations:**

Patient organizations will be invited to submit patient group input through an open call on EUnetHTA's website or via direct contact from assessment teams to European or national umbrella patient organizations or specific European or national patient organizations.

- *A modified version of the HTAi [Patient group Submission] Template (20) that includes questions from EUnetHTA HTA Core Model<sup>®</sup> will be prepared (EUnetHTA Patient Group Submission Template). Patient organizations interested in participating will be asked to complete the online and self-administered EUnetHTA Patient Group Submission Template.*
- *The EUnetHTA Patient Group Submission Template will be made available in different European languages.*
- *Patient organization(s) should collect and analyse patient input through their own channels (e.g. web survey, helpline analysis, social networking, focus group(s), patient records, one-on-one conversations with those who have experienced an intervention, patient stories, research studies).*

- *Patient organizations need to state their sources of funding in the questionnaire.*

**One-on-one conversation:**

This method allows asking more in-depth questions to one or more patients. The conversations are not primary research on patient perspectives, but performed to inform the assessment teams in the scoping phase. These conversations are usually held via telephone and should be conducted by an assessment team member who is experienced in interacting with patients.

- *The questions in the EUnetHTA Patient Group Submission Template can be used as a starting point for the one-on-one conversation.*
- *Patients will be provided with questions in advance and participate in telephone calls.*
- *The conversation should be in the local language of the patient when possible. Recording of the conversation may be helpful but not mandatory and consent from the patient needs to be obtained to do so. However, possible storage of recordings must be according to country regulations. Summary of the conversation(s) in form of written minutes should be provided to the patients for validation.*
- *English translation of the conversation summary would be helpful to inform the co-authors about the patient perspectives. This would also be helpful when presenting patient input in the assessment (see chapter 5).*

**Group discussion:**

The group discussions are not intended as primary research on patient perspectives, but are performed to inform the assessment teams in the scoping phase. In a group discussion, a moderator will guide the discussion. Group discussions may be appropriate in specific situations, but due to resource implications for both patients and EUnetHTA partners, this should be done only for a limited number of assessments.

- *The questions in the EUnetHTA Patient Group Submission Template can be used as a starting point for the group discussion.*
- *Recording of the discussion may be helpful but not mandatory and consent from the patients needs to be obtained to do so. However, possible storage of recordings must be according to country regulations. Summary of the discussion in form of written minutes should be provided to the patients for validation.*
- *English translation of the discussion summary would be helpful to inform the co-authors about the patient perspectives. This would also be helpful when presenting patient input in the assessment (see chapter 5).*

**Scoping e-meeting with patients:**

Individual patients or patient organization representatives should be invited to attend a scoping e-meeting where the EUnetHTA authors (and co-authors), and project manager are present.

- *The objective of the e-meeting should be to discuss all aspects listed in section 1 of this document (goals for patient input).*
- *The draft PICO should be made available for the individual patients or patient organization representatives for comment after the e-meeting, if agreed on by the assessment team.*

**Recruitment procedures:**

The Task Group discussed the following recruitment methods:

- *Patient organizations can be recruited through open calls on the EUnetHTA webpage or direct emails to patient organizations either at the EU or national level.*
- *For one-on-one conversations, recruitment of patients (or caregivers) can occur via EU or national patient organizations.*

- *For group discussions, recruitment of patients can occur via national patient organizations in order to allow the discussion to be conducted in the local language and reduce travel costs for patients.*
- *For scoping meetings with patients, recruitment of individual patients or patient organization representatives can occur via direct emails to patient organizations either at the EU or national level.*

## **5. How to make patient input visible in assessments**

The Task Group discussed the potential challenges of presenting patient input and patient perspectives in the final REA report. Viable ways to do this include:

- *Describe the method of patient input and when the input was gathered in the method section of the report*
- *Separate section or chapter on patient perspectives in the report based on answers from questionnaires, one-on-one conversations or group discussions, preferably supported by quotations from the patient-validated summaries.*

The Task Group recommends identifying commenters by status (patient or caregiver), age and/or gender (when relevant), country origin, and organizational affiliation (if applicable), but not by name.

## **6. Confidentiality, conflict of interest and compensation**

The Task Group discussed questions regarding confidentiality, conflict of interest and compensation and agreed on the following:

The EUnetHTA declaration of conflict of interest and confidentiality undertaking (DOICU) form needs to be completed and signed by every individual patient/caregiver/patient representative who participates in one-on-one conversations, group discussions or scoping e-meetings. All patient organizations that contribute in the open call for patient input need to provide information regarding their funding. The DOICU form can be shared among EUnetHTA partners.

Compensation procedures are outlined in the SOP "Compensation of external parties in Joint Action 3". The Task Group recommend reimbursement of travel costs for patients and/or caregivers. When possible, partner organizations might consider compensation of patient effort.

## **7. Evaluation**

The Task Group discussed the need for evaluation of the patient input in REAs. We agreed to create a questionnaire based on the one used for evaluation of patient engagement in Early Dialogue (ED). We also suggest that patients, caregivers or patient representatives receive feedback on how their contribution was used in the assessment after the assessment is finalized.

## **Summary**

The Task Group's preferred method of patient participation in REAs within JA3 is to collect input about patient perspectives during the scoping phase. We recommend using a patient submission template for organizational inputs, and/or one-on-one conversations, group discussions or participation in scoping e-meetings to obtain patient perspectives. All contributors need to fill out the DOICU form. Input should occur as early in the process as possible to inform development of the PICO. Table 1 gives an overview of contributions, compensation and confidentiality issues for preferred methods of patient input in REAs.

Table 1. Preferred methods for patient input in REAs within JA3

Approach	Patient and/or patient representative	Description of patient contribution & deliverables	Patient investment and compensation	Conflict of interest and confidentiality issues
<b>Open call for patient input</b>	<p>Patient organizations (representatives)</p> <p>Patient will have access to information publicly available, no additional confidential data will be shared.</p>	<p>General feedback from an organizational level; summarized view intended to be representative</p>	<p>Some investment for organizations; no compensation</p>	<p>Organizations provide info regarding funding and conflict of interest.</p>
<b>One-on-one conversation</b>	<p>Individual patients (living with the condition) or caregivers providing general or specific feedback; patients or caregivers may also act as patient / caregiver representatives</p> <p>Patient will have access to information publicly available, no additional confidential data will be shared.</p>	<p>Views are (mostly) individual</p> <p>Representatives may add a view that is intended to be representative</p>	<p>No travel costs if done via phone; compensation will be outlined in the SOP for compensation of external parties</p>	<p>Declaration of conflict of interest to be completed, any risk of conflict of interest will need to be assessed properly before patient engagement.</p>
<b>Group discussion</b>	<p>Individual patients (living with the condition) or caregivers providing general or specific feedback</p> <p>Patient will have access to information publicly available, no additional confidential data will be shared.</p>	<p>Views are (mostly) individual</p>	<p>Travel costs for individual patients or caregivers; compensation will be outlined in the SOP for compensation of external parties</p>	<p>Declaration of conflict of interest to be completed, any risk of conflict of interest will need to be assessed properly before patient engagement.</p>
<b>Scoping e-meeting participation</b>	<p>Individual patients (living with the condition) or caregivers providing general or specific feedback; patients or caregivers may also act as patient / caregiver representatives</p>	<p>Views are (mostly) individual</p> <p>Representatives may add a view that is intended to be representative</p>	<p>No travel costs; compensation will be outlined in the SOP for compensation of external parties</p>	<p>Declaration of conflict of interest to be completed, any risk of conflict of interest will need to be assessed properly before patient engagement.</p>

DOICU= conflict of interest and confidentiality undertaking form

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