**Minutes of the Patient and Consumer Involvement in EUnetHTA JA3 Meeting**
26 Jan 2018 | 11:00 – 17:00 CET
Room B232 08/120, Rue Belliard 232, Brussels

| Attendees |
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| Patient & Consumer Organisations: Kostas Aligiannis (EPF), Francesca Cattarin (BEUC), François Houyez (EURORDIS), Winne Ko (IDF) – by TC, Ancel.la Santos (HAI), Roberta Savli (EFA), Matteo Scarabelli (EURORDIS), Valentina Strammiello (EPF), Isabelle Manneh-Vangramberen (ECPC), Rebecca Moore (EIWH) – by TC, Yannis Natsis (EPHA)<br><br>EMA: Ivana Silva<br><br>European Commission: Paolo Catalani, Ioana Siska<br><br>EUnetHTA: Hannah Brühl (G-BA/Co-LP WP5), Sabine Ettinger (LBI-HTA/Co-LP WP4 Other Technologies), Maria del Carmen Sanchez Gonzalez (ISCII/WP2), Chantal Guilhaume (HAS/LP WP5), Iñaki Imaz Iglesia (ISCII/WP2), Michelle Mujoomdar (WP1), Ingvil Sæterdal (NIPHNO/LP), Anne Willemsen (ZIN/Co-LP Pharma) |

| Item | Final Agenda | Lead |
|---|---|---|
| 1 | Welcome & introductions | All |
| 2 | Welcome by DG SANTE | Ioana-Raluca Siska |
| 3 | Review of meeting objectives and involvement of European patient and consumer (P&C) organisations<br><br>Update on EUnetHTA Task Group | Michelle Mujoomdar |
| 4 | Presentation by P&C re: papers submitted to EC (to be presented at HTAN) & Discussion | TBD |
| 5 | Update from WP2 | Iñaki Imaz Iglesia & María del Carmen Sánchez González |
| 6 | Involvement of patients and consumers in the EMA: opportunities for collaboration + Discussion | Ivana Silva |
| 7 | WP5 – experiences to date and feedback/input on proposals to be further tested + Discussion | Chantal Guilhaume & Hannah Brühl |
| 8 | WP4 – experiences to date and feedback/input on proposals to be further tested + Discussion | Anne Willemsen & Sabine Ettinger |
| 9 | Next steps | Michelle Mujoomdar |

## 1. Welcome & Introductions

Participants were welcomed by EUnetHTA and introductions were made.

## 2. Welcome by DG SANTE

DG SANTE expressed that they were pleased to be able to host this meeting as it allowed participation from members of the team. The proposal will be published on 31 Jan 2018 along with the impact assessment report and the supporting studies that supported the development of the impact assessment report. The next meeting of the HTAN will be on 9 Feb 2018 and a dedicated meeting with the HTAN Stakeholder Pool is being planned for later this year.

## 3. Review of meeting objectives and involvement of European patient and consumer organisations & update on EUnetHTA Task Group

EUnetHTA reviewed the agenda – no additional topics were requested to be added. The meeting objectives were reviewed and agreed. In general, the meeting aimed to discuss how EUnetHTA engages with stakeholder, specifically Patients and Consumers (referred to as P&C), and how EUnetHTA could engage with the P&C pillar of the HTAN stakeholder pool going-forward. An additional aim was to discuss the opportunity to learn from and leverage existing model of collaborations (e.g., EMA). Finally, attendees agreed to engage in a dialogue regarding the experiences to date of WP4/5 in involving P&C and collect feedback on EUnetHTA's proposals for engagement.

An update on the EUnetHTA task group (TG) on P&C involvement was provided. The last meeting EUnetHTA had with P&C organisations was in March 2017 and attendees represented only a subset of organisations within the HTAN stakeholder pool. At that time, involvement of P&C in EUnetHTA's activity was sporadic. The EUnetHTA Executive Board requested that a cross-work package (WP) TG on the topic of involvement of P&C and Health Care Providers (HCP) be established. A cross-WP perspective would promote consistency between WPs in the establishment and application of principles for the involvement of P&C and HCPs. The resulting task group was established in September 2017 and comprises representatives from WP1, WP2, WP4, and WP5. The TG is operational in nature and the scope is involvement of P&C/HCP at the project level. At this time, the TG has prioritised the development of procedures to involve P&C. This TG will support the development of such procedures. Another cross-WP TG was established at the same time and is reviewing EUnetHTA policy and procedure for handling conflict of interest. WP1/4/5 are represented on the COI TG.

Discussion/Questions:

- It was mentioned that there are groups with whom EUnetHTA has engaged and will likely engage with in the future who aren't part of the HTAN Stakeholder Pool. How can those organisations engage with EUnetHTA? The preference for EUnetHTA is to engage with members of the HTAN Stakeholder Pool as it ensures that all organisations are receiving similar information at the same time. There isn't a limit to the number of organisations who can join pool. Those organisations meeting eligibility criteria are encouraged. It was noted that some organiations may not have the capacity to participate.
- Action: It was noted member/umbrella organisations may wish to explore with their members who would be able to join the stakeholder pool as individuals organisations.
- COI was noted as a topic that is of interest to the P&C group and members expressed an interest in also being updated on the status/progress of the COI TG.
    - Action: MM to provide updates.

## 4. Presentation by P&C re: papers submitted to EC (to be presented at HTAN) & Discussion

The P&C pillar of the HTAN Stakeholders Pool were asked by the European Commission (EC) to prepare three documents to support the development: 1) principles for engagements of P&C in HTA; 2) Criteria for prioritisation of technologies for joint HTA; and 3) eligibility rules for patients and consumers organisations. The third document is in development; however, the first two documents have been endorsed. It was noted that while the documents were formally endorsed by all organisations, there isn't consensus on, for example, the definitions of the principles. [Refer to slide deck for further detail]

Briefly, the principles are: Inclusion, Legitimacy, Transparency-Visibility, Publicity, Relevance, Appeal/Revisability, Responsibility, and Enforceability.

Discussion/Questions related to the first paper:

- There was a request for clarification on the principle of appeal/revisability (elaborated as: "There should be the possibility to challenge the decision"). It was noted that the use of the word "decision" poses some difficulty as decisions are made at a national level. Though this is a proposal for a post-2020 scenario, a substitution of decision for "conclusion" may wish to be considered. Such a challenge could be based on the emergence of new evidence or a procedural challenge (e.g., how patient input was used). Some national HTA systems have appeal processes and these processes may wish to be further analysed.
- Additional questions included centered around the application of such a principle. For example, which patient has the right to challenge? Would it be single patient? A patient organisation?

Distinct prioritisation criteria for pharmaceutical products and non-pharmaceutical products were proposed in the second paper (before the criteria listed in the proposal became public). Briefly, the criteria for pharmaceutical products include:

- Advanced Therapies Medicinal Products (orphan or not)
- Orphan Medicinal Products (not all: for very rare diseases of when not all MSs have expertise)
- Others

All pharmaceutical products expected to be assessed (and summarised in the above categories as general hypothesis) would need to meet at least 3 of 5 of the following:

- Complex and disruptive technologies
- Benefits/risks is not clear
- High budget impact
- Significant increased use (e.g. off label)
- Antimicrobiotical products targeting resistant strains

The criteria for non-pharmaceutical products include:

- Medical devices: invasive and/or implanted ones
- Complex surgery
- Disruptive technologies with impact on organisation of healthcare
- Connected devices or apps implying privacy risks or substituting HCP for diagnosis
- Prevention programmes requiring cross-border actions

For both pharmaceutical and non-pharmaceutical products, the P&C group proposed that "obsolete" technologies should also be considered for topics for joint HTA. This could inform a disinvestment decision.

Discussion/Questions related to the second paper:

- It was noted that the criteria were developed via a brainstorm and are based on criteria that are known to be employed by other prioritisation systems.
- There was a question regarding if the group had considered a criterion such as 'unmet medical need'. It was noted that, to some degree, unmet medical need is implied by most of the criteria. It was also noted that definitions of terms such as unmet medical need and disruptive technologies can differ between regulators and HTABs and between HTABs.
- It was noted that for non-pharmaceutical technologies, an area of focus might be those technologies for which clinical study data are required for CE mark as part of the new medical device legislation.

- Regarding re-assessment of potentially obsolete technologies, it was asked if this was best done at an EU-level or if this was something that might be more context sensitive and more appropriate for national level HTA.

The third and final document is still be drafted and purports to suggest eligibility rules for patients and consumers organisations within permanent cooperative structure). A draft list includes:

- Not-for-profit
- Legitimacy (legitimate claim to represent P&C across the EU)
- Legal entity (legally established in the EU – with an EU focus and independent)
- Structure (governing bodies elected by their members – corporates excluded)
- Accountability (adequate means and procedures to consult and communicate with members)
- Transparency (registered status, disclosure of mission and objectives, list of members (governing bodies and geographical spread), sources of funding, annual financial statement, code of conduct for relations with funders)

Other criteria being considered include:

- Diversity of funding (financial conflict in interests? Thresholds, diversification?
- Geographical spread (one quarter of member states; 25%: 7 countries)?
- Disclosure of material and immaterial benefit
- Other organisations (should we enlarge the consultation)?

Discussion/Questions related to the third paper:

- EMA offered to share experiences or additional detail, if required, regarding eligibility criteria for organisations and how the application of the criteria may differ depending in which activity the P&C is participating. Briefly, if an organization has more than 20% annual budget coming from industry funding, then the funding must come from at least three different companies for an organization to be eligible. Such threshold and diversification rules differ between HTA agencies.
- Action: EUnetHTA will develop scenarios and discuss with EMA to better understand how COI would be managed in such scenarios.
- There was a comment that, where possible, EUnetHTA may wish to consider following the same criteria as EMA to avoid a situation where P&C are able to participate in one process, but not the other. It was clarified, that as EUnetHTA isn't a legal entity, the national rules of the participating HTABs prevail and as such, the EUnetHTA procedures may differ from that of EMA. In addition, while EUnetHTA seeks to leverage experience and learning regarding EU-level stakeholder engagement from EMA, EUnetHTA is committed to developing procedures that are fit-for-purpose for HTA and meet the needs of its partners.

**5. Update from WP2**

An overview of EUnetHTA's stakeholder and the different modes of involvement was provided by WP2. In addition to the stakeholder groups represented in the HTAN Stakeholder Pool (P&C, HCP, Payers, and Industry), EUnetHTA also engages with regulators, other HTABs, academia, and scientific organisations.

In general, stakeholders are foreseen to engage in EUnetHTA's work by participating in specific WP activities, responding to public consultations, providing input at the level of the HTAN, and participation in the EUnetHTA Forum.

With respect to the EUnetHTA Forum, the dates for the next Forum have been confirmed for 25 May 2018. WP2 shared activities that are in-progress or planned, these include use of the Extranet which represents a platform for information exchange and potentially, a collaborative work space.

Discussion/Questions:

- EUnetHTA emphasised interest in hearing suggestions for topics to be covered at the Forum and ideas regarding format from P&C (as well as other stakeholder group)
- There was a suggestion to consider extending the Forum to the day after; however, this might not be feasible because that would be a Saturday. This could be something to be considered in the future. In general, it would be efficient and beneficial to consider opportunities to leverage planned meetings (e.g., HTAN meetings or perhaps PCWP meetings) to extend to cover EUnetHTA-specific items.
- A number of materials that would be of interest to P&C groups to facilitate involvement could be posted on the Extranet – including Letters of Intent or a link to the HTA Database with completed HTA reports.

**6. Involvement of patients and consumers in the EMA: opportunities for collaboration + Discussion**

EMA provided an overview of how the Agency involves P&C organisations and individuals as well as HCP in their work. Four frameworks for engagement have been established out outline engagement with P&C, HCP, industry, and academia. The focus of the meeting was on P&C.

EMA has an Individual expert database which includes over 200 patients. Individuals can register for this database and indicated their area of expertise/interest. If they are contacted to be involved in a scientific activity, they are asked to provide information on COI.

Currently, EMA engages with 35 P&C organisations. Organisations can either contact EMA to become involved or if EMA becomes aware of organisations, they may reach out. Organisations are requested to register and need to provide an annual assessment of their funding as well as comply with a number of requirements. Of the 35 organisations, 20 are represented on EMA's Patient and Consumer Working Party (PCWP). The selection of the 20 organisations is an executive decision and is informed by the mandate of the agency. The term for an organisation is three years; however, there is the opportunity for the term to be renewed. The matters discussed at the PCWP or not product-specific. The PCWP is co-chaired by member of EMA staff and one elected representative from the P&C organisations.

EMA engages with individual patients, their carers, as well as patient representatives. Patients can represent an organisation or can represent their individual perspective. Patients can represent their community (e.g., on EMA's management board or scientific committees), their organisations (e.g., when providing feedback to EMA's consultations or attending workshops organised by EMA), or patients can act as individual experts (e.g., during scientific advice/protocol assistance procedures, ad-hoc expert groups, or when reviewing documents requested by a scientific committee).

An annual training is held by EMA for P&C. The next training is scheduled for Nov 2018 and there may be a possibility for a EUnetHTA representative to join. Action: EMA will also discuss with the PCWP and HCPWP the interest in including a session on HTA within the training.

Discussion/Questions:

- EMA shared that the need for patient(s) to be involved in a scientific advice/protocol assistance identified at the beginning of the activity and is generally standard when there

is a clinical question. If a developer is asking a question of a non-clinical nature (e.g., quality), a patient may not necessarily be engaged.

- EMA explained the concept of 'expert witness', where, exceptionally, a patient (individual or representative) or a health care provider may be involved despite having an identified conflict. For example, such a situation may arise during evaluation of orphan medicines.
- EMA distributes a survey to the scientific advice team gather their feedback on the involvement of patients in terms of the value it added to the advice and to the procedure itself. Action: EMA to share survey with EUnetHTA.
- In response to a question from EUnetHTA, EMA shared that for most scientific advice projects, a single patient or perhaps two are involved. A single perspective has been found to be valuable.

**7. WP5 – experiences to date and feedback/input on proposals to be further tested + Discussion**

WP5 provided an overview of the three pathways for Early Dialogues (ED) involving HTA bodies, with an emphasis on the two pathways involving EUnetHTA – i.e., the EUnetHTA multi-HTA procedure and the EMA-EUnetHTA Consolidated Parallel procedure (PCC) [See slides for additional detail]. WP5 has experience involving patients in three EDs. In one instance, an interview was conducted by the ED coordinator and rapporteur with one patient represented via an EU patient organisation. Feedback from the participating HTABs was positive and the patient's feedback was considered during the preparation of common and individual HTAB positions. Learnings from this experience included:

- a preference for more than one patient coming from different countries
- a minimum time to review Briefing Book was defined and needs to be factored in for future projects
- patient input should be visible in the final recommendations
- a number of operational issues related to contracting and COI were identified

For two PCCs, patients were recruited by EMA and participated during the face-to-face meetings with the company. This approach was less satisfactory to HTABs as there is a strong preference to engage with patients ideally as HTABs are developing their list of issues and definitively as their positions are being developed.

From a WP5 perspective, patients' perspective is essential at the time of forming their advice as patients contribute their experience of living with the condition. They are also able to advise on the symptoms and adverse events that have the greatest impact. Patient representatives with knowledge of HTA or of clinical trials may be able to contribute to aspects related to the clinical development plan. Whether involving individual patients or representatives from a patient or consumer organisation, WP5 stressed the importance of transparency with respect to potential COI and adherence to confidentiality requirements. As EUnetHTA is not a legal entity, the rules that are followed for involvement of patients and experts are those of HAS, the LP for WP5.

Three approaches for involving patients and consumers that are or will soon be tested in WP5 were described:

1. Individuals patients living with the condition can provide feedback. This would be conducted in the form of an interview in the local language of the patient by the respective HTAB (Note: the language would need to be one spoken by one of the HTABs participating in that ED). The minutes would be translated to English and provided to the other HTABs. For this scenario, the risk of issues arising due to COI or confidentiality is considered to be low.
2. A patient representative would provide general feedback and comment on issues

identified by HTABs. Similar to scenario one, an interview in the patient representative's local language would be conducted and the minutes would be translated to English and provided to all HTABs participating in the ED. For this scenario, the risk of issues arising due to COI or confidentiality is considered to be high.

3. A patient representative would provide general feedback, comment on issues identified by HTABs, and also address specific questions or issues identified by HTABs. In addition to an interview that would be conducted in English by the EUnetHTA ED scientific coordinator and rapporteur, the patient representative would attend the e-meeting between HTABs where the list of issues is discussed as well as the F2F meeting with the company. For this scenario, the risk of issues arising due to COI or confidentiality is considered to be high.

Discussion/Questions:

- It was noted that in the current procedures, companies must agree to share the briefing book with patients. Not all companies agree to this. A suggestion was made for WP5 to consider making this part of the standard procedure so that companies interested in participating would know that this was a requirement rather than optional.
- A suggestion was made to consider having or participating in annual training that could help to demystify the process for the patients.

**8. WP4 – experiences to date and feedback/input on proposals to be further tested + Discussion**

WP4 Co-LPs for Pharmaceuticals and Other Technologies shared the WP4 goals for patient involvement in joint and collaborative assessments. WP4 aims to:

- elicit patients' views on aspects regarding their condition and currently available treatments
- understand how their conditions impacts upon quality of life
- gather information on outcomes that are important and relevant from a patient's perspective
- to gain insight into issues that are of an ethical or social nature (to inform the ethical and social checklist of the relative effectiveness assessment)

Four preferred approaches for involving patients were described:

1. Open call for patient representatives to be involved in assessments
    - A call would be placed on the EUnetHTA website and/or distributed to the participating P&C organisation of the HTAN stakeholder pool.
    - Patient organisations would be asked to complete a modified version of the HTAi questionnaire (Note: three template questionnaires have been developed)
    - This would allow for a general patient perspective to be gathered and as it would be done early in the process, it would inform the development of the PICO (population, intervention, comparators, outcomes) table.
    - This approach is currently being tested for a joint assessment of continuous and flash glucose monitoring devices.
2. Semi-structured interview
    - The HTAi questionnaire would be used as a starting point and adapted to include some questions from the EUnetHTA HTA Core Model.
    - Individual patients would be recruited via EU patient organisations. Patients would be provided with the questions in advance and will participate in a telephone call.
    - The call will be recorded, transcribed, and provided to the patient for validation.
    - This approach was used in two joint assessments of pharmaceuticals. It was found to be more helpful when done early to inform the scope of the EUnetHTA report.
    - It allowed interviewers to ask more in-depth questions to patients.

3. Scoping e-meeting without the manufacturer
    - For this approach, patient representatives attend a scoping meeting where the manufacturer is not present – only the EUnetHTA authoring team and coordinator.
    - The draft PICO will be available for the patient representatives to comment
    - This approach has been taken for one collaborative assessment of other technologies and one further is planned.
4. Focus group
    - A final approach was presented and it was noted that focus groups may be appropriate in specific situations, but due to resource implications for both patients and EUnetHTA partners, this could only be done for a limited number of assessments.
    - A moderator would guide a group of individual patients using a semi-structured interview approach. The questions can be based on the HTAi questionnaire or other tools and could be adapted to include questions from the EUnetHTA HTA Core Model.
    - The discussion is recorded, transcribed, and analysed
    - The focus group would take place at the beginning of the scoping phase
    - Recruitment of patients would be done via national patient organisations in order to allow for the focus group to be conducted in the local language and reduce costs of travel for patients.
    - A focus group was conducted for one collaborative assessment and two are planned for an on-going joint assessment.

It was recognised by WP4 that the input from a patient perspective should be visible in the assessment.

Discussion/Questions:

- It was mentioned that P&C organisations find the timing to involve patients very limited and request that EUnetHTA provide as much information as possible when making requests to facilitate recruitment.
    - Action: WP4 to send draft email template to P&C organisations.
- It was discussed the EUnetHTA Topic Identification, Selection and Prioritisation (TISP) group that was established by WP4 should discuss how topic proposals raised by, for example, P&C organisations should be managed.
    - Action: TISP members present at the meeting will raise this with the larger group
- A suggestion was made to post on the EUnetHTA website a table which would describe in which assessments and during which stages patients were involved.
- It was flagged by both P&C participants as well as EUnetHTA participants that language barriers are a challenge. Where possible, interviews in patients' local languages are preferred. With respect to the HTAi template, it should be possible to have it translated into other languages by HTABs (Note: the translation of the template into French was done by HAS).
    - Action: to be discussed with HTAi Patient and Citizen Interest Group translation of the HTAi template by select HTABs [TG members]
- All participants acknowledged that providing training for patients is essential in ensuring that patients who participate in EUnetHTA activities will be comfortable with the procedure, thereby allowing them to contribute optimally. In addition, training can provide an opportunity to build trust between participating patients and EUnetHTA.
- Final timelines for joint and collaborative assessments are currently available within the project plans that are posted on the EUnetHTA website.

- Action: WP4 Project Managers will share draft timelines with the P&C organisations in the initial correspondence.
- Action: Timelines for EDs (Multi-HTA and Parallel Consultation) will be posted on the EUnetHTA website (week of 5 March).

**9. Next steps**

Participants were thanked for their active contribution to the meeting. EUnetHTA will use the feedback provided and further develop the approaches. WPs are asked to share with the P&C organisations how feedback was considered.

| Action Points | Responsible |
|---|---|
| Member/umbrella organisations may wish to explore with their members who would be able to join the stakeholder pool as individuals organisations. | P&C organisations |
| Provide updates on COI TG to P&C organisations | Michelle |
| EUnetHTA will develop scenarios and discuss with EMA to better understand how COI would be managed in such scenarios. | Chantal/Hannah |
| EMA will discuss with the PCWP and HCPWP the interest in including a session on HTA within the training. | Ivana |
| EMA to share survey that is sent to Scientific Advice staff within EMA with EUnetHTA. | Ivana |
| WP4 to send draft email template to P&C organisations. | Sabine |
| TISP members present at the meeting will raise this with the larger group | Anne/Sabine/Chantal |
| Discuss with the HTAi Patient and Citizen Interest Group translation of the HTAi template by select HTABs | TG members |
| WP4 Project Managers will share draft timelines for joint and collaborative assessments with the P&C organisations in the initial correspondence. | WP4 |
| Timelines for EDs (Multi-HTA and Parallel Consultation) will be posted on the EUnetHTA website (week of 5 March). | WP5 |