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Advanced EUPATI training for increased patient involvement in Health Technology Assessment (HTA) → HTA4Patients

MILESTONE 1 'Kick-off alignment workshop'

Work Package	1, Project Management, Coordination and Infrastructure
Lead	EUPATI
Means of verification	Workshop minutes
Authors	Silvia Scalabrini, Maria Dutarte
Month and Date of delivery	M2, April 2023

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Executive Summary

This milestone reports on the EUPATI HTA4Patients Kick-off Alignment Workshop which took place at the Brussels Life Science Incubator Centre in Brussels on March 13-14, 2023.

This report includes a description of the event and its objectives, along with summaries of the various presentations, including the discussions had during the Q&A sessions. Two group activities were carried out on the second day of the workshop, which are also described. The Annex includes five attachments: the signed list of participants; a photograph of the participants; the workshop agenda along with all the supporting material circulated prior to the event; the EUPATI slide-set for the event' and the presenters' slide-sets.

General Information

EUPATI submitted its project proposal as a single applicant to a call by the European Health and Digital Executive Agency as part of a larger initiative called EU4Health Programme. The kick-off alignment workshop was the first general meeting of the HTA4Patients project, which was focused on formally launching the 36-month project, setting the context within which the project activities were developed, defining the operative framework along with next steps and actions to be taken in order to achieve its objectives.

The workshop was organised by EUPATI and it served the following purposes:

1) Provide the project's overview to all the stakeholders involved
2) Provide some key background information relevant to the project implementation and monitoring
3) Introduce the governance structure as well as establish lines of communication
4) Present the project's objectives, milestones and deliverables within each Work Package (WP)
5) Describe project monitoring and management processes including reporting, amendments and visibility of the project

List of Participants & Affiliations

Name	**Affiliation**	**Day of attendance**
Maria Dutarte	EUPATI	Day 1-2
Silvia Scalabrini	EUPATI	Day 1-2
Bojan Cigan	EUPATI	Day 1-2
Vaida Jureviciene	EU4 Health/Health and Digital Executive Agency	Day 1-2
Valentina Barbuto	DG Sante'	Day 1
Anke-Peggy Holtorf	Health Technology Assessment International	Day 1-2
Valentina Strammiello	European Patients' Forum, EPF	Day 1
Julie Spony	European Patients' Forum, EPF	Day 1

Petra Adamkova	EUPATI Fellow	Day 1-2
Peter Lems	National EUPATI Fellow	Day 1-2
Birgitta Termander	EUPATI Fellow	Day 1
Lotte Klim	EUPATI Fellow	Day 1-2
Paola Kruger	EUPATI Fellow	Day 1-2
Derick Mitchell	Irish Platform for Patients Organisations Science and Industry	Day 1
Wolf See	EUPATI Editorial Board	Day 1-2
Margareth Galbraith	Haute Authorite de Sante	Day 1-2
Julia Chamova	ISPOR, the Porfessional Society for Health Economics and Outcomes Research	Day 1-2
Anne Willemsen	European Network for Health Technology Assessment, EUnetHTA	Day 1-2
Julien Delaye	European Organisations for Rare Diseases, EURORDIS	Day 2

Workshop minutes

DAY 1

The first part of the workshop took place from 1pm to 5pm on Monday 13th March. The agenda is reported below:

13.00	Welcome & Opening remarks	Maria Dutarte
13.15	Who is who in the consortium	Silvia Scalabrini
13.45	'HTA warm up' – quick recap of the key concepts	Anke-Peggy Holtorf
14.15	What is the new HTA Regulation and what are EUnetHTA 21 experiences with patient involvement?	Anne Willemsen & Valentina Barbuto
14.45	Q&A and Open Discussion	All
15.15	Break	
15.45	Project overview, work packages and timelines	Silvia Scalabrini
16.00	Project implementation requirements	Vaida Jureviciene
16.25	Q&A	All
16.50	Introducing DAY 2 agenda	Maria Dutarte

17.00	End of DAY 1, checking in at hotel (if relevant)	

Maria Dutarte, EUPATI's Executive Director, welcomed the participants to the event, emphasising the importance of this worthwhile project proposal in the field of HTA. She mentioned the EUPATI Code of Conduct and Ethical Framework (https://eupati.eu/about-us/values-principles/) which outline the working culture and spirit within EUPATI as well as the ethical ground rules. These will act as guiding principles throughout the project implementation.

Participants were invited to use their social media channels and post about their participation. Further, they were also asked to introduce themselves in order for the group to familiarise with each other and understand their background. In addition to state their names and affiliations, participants were invited to elaborate on their previous experiences with HTA as well as what patient education meant to them in their everyday practice.

Anke-Peggy Holtorf, Project Coordinator of the Patient and Citizen Involvement in HTA Interest Group at HTAi, provided an overview about HTAi and their mission, the Patient and Citizen Involvement in HTA Interest group and their objectives. The second part of her presentation offered a detailed description of the definition of Health Technology Assessment (HTA), what value is and how it can be defined including HTA in practice. The practical example opened up the opportunity to outline what patients' contribution to the HTA process may be. An extensive overview of examples of patient involvement in HTA (using submission templates) was provided along with examples of patient involvement in HTA along the HTA process. In particular, an example related to the National Institute for Health and Care Excellence (NICE), UK, as to how the process develops and the related timeline was shared. For detailed information, the full presentation is available as Attachment 5.

Valentina Barbuto, the Policy Officer within Sante' C2, presented the new Regulation (EU) 2021/2282 on HTA. The key principles of the Regulation were described highlighting that ultimately Member States would remain responsible for drawing conclusions on adding value for their health systems and taking decisions on pricing and reimbursement. A detailed description related to the Member States Coordination Group on HTA was provided including the various sub-groups and the governance infrastructure. The timeline for the implementation for the HTA Regulation was also outlined: it was adopted in December 2021, it came into force in January 2022 whereas the date of application will be January 2025 (less than two years left for the application). The implementation phase will last five years (until January 2030) when the Joint Clinical Assessment will be implemented in full scope. For detailed information, the full presentation is available as Attachment 5.

Anne Willemsen, Senior Project Manager for EUnetHTA 21, JCA Secretariat, outlined and discussed the EUnetHTA experiences with patient involvement to date. Firstly, the difference between HTA and the Regulatory process were described, which were also connected to the new HTA Regulation division of responsibilities. The Joint Scientific Consultation (JSC) and the Joint Clinical Assessment (JCA) were defined and their aims clarified along with an overview of the EUnetHTA HTA Core Model. Following on from this, the suggested processes for external experts and stakeholder involvement in JSC and JCA were outlined, including how and at what stage of the process. Lastly, the presentation provided some practical suggestions as to how to get ready for the EU-wide HTA processes. For detailed information, the full presentation is available as Attachment 5.

Q&A Session

A **Q&A** session followed these presentations, a summary of some of the points raised is listed below:

- What is the scope of the Joint Scientific Consultation? This is currently under debate and discussions are ongoing; officially that's not in the scope of the Regulation;
- Conflict of interest: what approach will be followed? This has not been developed yet but of course it is a high priority task to be developed over the following months;
- It is important to make the topic and this area of focus 'attractive' in terms of being able to convey the importance of the area to people. It is fundamental to work with as many patient advocates as possible to drill down the key issues and understand where contributions are possible, what kind of processes can I influence? The challenge is to get people involved and get them to understand HTA and what it involves;
- It will be very important to have very clear practical examples showing how change can be brough about by patients. This helps patients to understand how they can influence the area – get a medicine approved and how other patients may then be able to benefit from it. Examples should be included along with the theory;
- A useful source including examples could be an HTAi project on patient involvement in HTA, this project is underway. Impact is not reimbursement or not rather getting to the best decision in the interest of society;
- Is it optional to involve patients or is it a legal requirement? In the Regulation there is an obligation to involve experts/patients;
- HTA bodies are going to be doing the assessments however the Stakeholder Network remains important in issues such as those related to methods; there is an obligation to make it work from the Commission's side so a lot of thinking has been done and will be done to ensure that this happens;

- EUPATI offers a generic and non-disease specific educational programme: this aspect should be considered when developing the HTA related content as it is not possible to address all existing HTA process in place across Europe. Ideally, it would be about getting people to understand how the process works and the practicalities involved.

Silvia Scalabrini, the EUPATI Project Coordinator, outlined the governance structure, which includes a Project Management Group as well as a Project Expert Panel. The EUPATI Team is ultimately responsible for the whole project delivery, monitoring and reporting. The four WorkPackages (WPs) were introduced and described:

1) WP1: Project Management, Coordination and Infrastructure (M1-M36)
2) WP2: E-learning Training Course (M1-M30)
3) WP3: Online Training Sessions (M13-M36)
4) WP4: Communication, Dissemination, Outreach and Sustainability (M1-M36)

A Gantt chart was also tabled during the event, which included a timeline in relation to Milestones and Deliverables. For detailed information, the full presentation is available as Attachment 4.

Vaida Jureviciene, the Project Officer from the Health and Digital Executive Agency (HaDEA), explained the grant management process including the various steps involved as well as the requirements related to the reporting process throughout the project duration. Aspects related to communication, dissemination and visibility were also described, highlighting the importance of the project visibility and the compliance with the funding display requirements. For detailed information, the full presentation is available as Attachment 5.

DAY 2

The second part of the workshop took place from 9am to 12pm on Tuesday 14th March. The agenda is reported below:

9.00	Group activity 1: Target audience mapping	Anke-Peggy Holtorf
10.15	Break	
10.30	Group activity 2: Forming the 'State of the art review' working group	Anke-Peggy Holtorf and Lotte Klim
11.40	Closing remarks and next steps	Maria Dutarte

Day 2 of the workshop focused on two group activities, whose objectives were to start a conversation related to two distinct aspects: 1) **Group activity 1**: the target audience of the e-learning course and the twelve online training sessions 2) **Group**

activity 2: 'state of the art review': what information would the new training material cover?

1) **Group Activity 1**: What is the **target audience** of the e-learning course and of the online training sessions? Who would we be talking to and engage with?
   The points made for the **online training sessions** related to participants' suggestions and ideas about who the audience of these should be. These included: EUPATI Fellows and more experienced patient advocates in general. Geography: EU MSs with established HTA processes, to include two lower GNI countries. Disease areas: oncology+ATMPs but potentially also others as coming up for HTA. It will be important to contact the HTAR Coordination group. Age: that can be a mix although some thought was given in relation to engagement activities with young people. The general assumption would be that young people do not necessarily want to engage in a traditional e-learning course rather finding new and innovative and engaging learning methods could be the best bet. However, this could be outside of the specific scope of the project.

   The points made for the **e-learning course** related to participants' suggestions and ideas about who the audience for this should be. These included:
   EUPATI Fellows, patient experts in general, beginners and interested experts. Geography: all EU countries and beyond. Disease area: open so anyone can read through the content on the EUPATI Open Classroom (free for everyone to access) as per EUPATI's underlying philosophy.

   Key success factors were also discussed as a way to imagine and reflect concretely on what 'success' would look like in relation to the project. the following factors were identified:
   - Patients (ATMP+oncology) understand HTA
   - Promoting and raising awareness to patient organisations and HTA bodies (with the possibility to organize an introductory webinar to the latter)
   - HTA bodies find patient involvement relevant and trained people can improve HTA processes
   - Increase capacity to provide input to the Coordination Group and in the HTAR
   - A set percentage of participants being active 'HTA Ambassadors'
   - EUPATI becomes a matchmaker between HTA bodies and trained patients
   - Sustainability of the outputs will continue well beyond the project duration (36 months)

2) **Group Activity 2:** State of the Art review: EUPATI already has a HTA module on its Open Classroom(https://learning.eupati.eu/local/coursecatalogue/index.php?cate

goryid=7), which includes a variety of topics. New information related to the HTA Regulation and key points will need to complement the existing module. New topics and specific information were suggested in relation to each of the five courses included in the EUPATI HTA module. Participants' ideas and discussion points are reported below next to each course within the module:

- **EUPATI Course 1, Introduction to HTA**: quick introduction to pharma R&D; value of patients' input in both EU and national HTA processes; visual roadmap including structure processes and stakeholders; description of all stakeholders; focus on why patients should know about HTA even if they don't necessarily participate in HTA; new HTAR; lifecycle (where does HTA fit in?); different remits (regulatory vs HTA vs pricing/reimbursement decision-making) including the meaning of patient involvement in each of them; marketing authorization, HTA pricing reimbursement, EU HTAR overview, why and what is there for me so benefits for patients; general knowledge about R&D process and regulatory framework, introduction on why to involved in HTA, roadmap

- **EUPATI Course 2, HTA bodies and principles**: principles of market access; remit of HTA; where HTA bodies fit in the development cycle; new EU Regulation; EMA process and HTA process; economic perspectives (simple method and elaborate method); HTA process (clinical value, economic perspective practice); new HTAR and this fits into the MS HTA processes; types of HTA bodies in Europe (similarities and differences); main course on general HTA principles and sub-course on national/regional processes; survey and map of HTA bodies in EU and beyond if possible, basic modus operandi, differences in approaches; examples of different national bodies/patient involvement tasks; what is HTA.

- **EUPATI Course 3, HTA and evaluation methods, quantitative**: HTA in practice, examples; EU specificity with similarities and differences

- **EUPATI Course 4, HTA and evaluation methods, qualitative**: EU MS specific contexts including similarities and differences; patient involvement in R&D (assessment is patient experience and patient preferences)

- **EUPATI Course 5, Patient involvement in HTA**: roadmap re EU involvement and at the national level; roadmap HTA with patient involvement objectives, type of patients, expected contribution and type of patients; roadmap with real world examples; practical tips/tools on where to find helpful glossaries, databases to sign-up etc; what is expected of your participation, what type of questions will be asked and not asked; guiding questions patients could ask to HTA bodies to understand their role/get familiar with what is expected from them; how patient input is used in final product/report; example of EU HTA report, sharing extracts of patients' input; why is it useful to be involved (patients'

perspectives); practical examples; how to answer to patient involvement if you are a proxy/carer; use of case studies to show how patient involvement has been included so far and actual results; EU level HTA process, national level HTA process; patient relevant HTA questions/RQs are relevant across clinical trials, regulatory processes and HTA; patient involvement at the organizational level; how to respond to templates (practical exercises); a simulation of involvement as part of the course; practical things how patients can participate; more practical; example of involving patients (how the procedure was done, one patient submission); conflict of interest; how to fill in conflict of interest forms and what info HTA is looking for; confidentiality JSC/JCA; how to collect patient experience legally (GDPR)

- **Extra suggestions which would not fit within the current module structure**: RWE in HTA, RCT RWD (?); rare diseases, challenges and differences; patient involvement in economic assessment; approaches and consequences of different drivers: cost effectiveness and clinical effectiveness; course Zero: what happens before HTA?; new regulation; HTAR perspective included in all courses; 1 whole course on HTAR, what is it, why is it here, how is it implemented, overview of the whole HTA process including HTAR; therapies and medical devices examples, perhaps show differences and similarities;

Both Group Activities discussions were seen as fundamental in terms of supporting EUPATI's thinking on these tasks, the practicalities involved and what the next steps may be.

The event came to an end at 11.45am. EUPATI thanked all the participants for attending and for actively contributing to the event. They were informed that further information regarding next steps and concrete activities would be shared in due course.