BACK TO LOG OF ACTIVITIES

Annex:
Patient engagement activities and methods of engagement

This annex summarizes patient engagement activities along the medicines' lifecycle and most commonly used methods of engagement. For more exhaustive information, see the reference document Raising awareness on managing competing interests in a multi-stakeholder environment: Guidance to patients and engaging stakeholders

TABLE 1: **Patient engagement activities along the medicines' lifecycle**[1]

Phase of the medicine lifecycle	Types of activities	
Research priorities	Setting research priorities	Gap analysis
		Early horizon scanning
		Matching unmet needs with research
		Defining patient relevant added value and outcomes
Research priorities and Planning	Protocol synopsis	Design and target population
	Protocol design	Relevant endpoints
		Benefit/Risk balance
		Inclusion/exclusion criteria
		Diagnosis procedures
		Quality of life and patient reported outcomes
		Ethical issues
		Data protection
		Mobility issues/logistics
		Adherence measures
	Patient Information	Content, visual design, readability, language, dissemination
	Informed consent	Content, visual design, readability, language
	Ethical review	

[1] List of patient engagement activities along the medicines' life-cycle adapted from the EUPATI roadmap: Geissler, J., Ryll, B., di Priolo, S. L., & Uhlenhopp, M. (2017). Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap. Therapeutic Innovation & Regulatory Science, 51(5), 612–619. Available at: https://doi.org/10.1177/2168479017706405

TABLE 1: **Patient engagement activities along the medicines' lifecycle**[1]

Phase of the medicine lifecycle	Types of activities	
Research priorities and Planning	Trial steering committee	Protocol follow up
		Improving access and adherence
	Information to trial participants	Protocol amendments
		New safety information
	Investigators meeting	Trial design
		Recruitment
		Challenges and opportunities can trigger amendments
	Data & Safety Monitoring Committee	Benefit/risk; drop-out issues; amendments
	Study reporting	Summary of interim results and dissemination in patient community
Dissemination, communication, post-approval	Regulatory Affairs	For relevant activities see Raising awareness on managing competing interests in a multi stakeholder environment: Guidance to patients and engaging stakeholders
	HTA	For relevant activities see Raising awareness on managing competing interests in a multi stakeholder environment: Guidance to patients and engaging stakeholders
	Post-study communication	Contribution to publications; dissemination of research results to patient community/professionals

[1] List of patient engagement activities along the medicines' life-cycle adapted from the EUPATI roadmap: Geissler, J., Ryll, B., di Priolo, S. L., & Uhlenhopp, M. (2017). Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap. Therapeutic Innovation & Regulatory Science, 51(5), 612–619. Available at: https://doi.org/10.1177/21684790177064405

TABLE 2. **Types of EMA engagement activities[2] and DOI requirement**

Phase of the medicine lifecycle	Types of activities	DOI required
Pre-submission	Scientific Advice (SA) and Protocol Assistance (PA) (https://www.ema.europa.eu/en/human-regulatory/research-development/scientific-advice-protocol-assistance)	Yes
	Parallel consultations with EMA and HTA bodies (https://www.ema.europa.eu/en/human-regulatory/research-development/scientific-advice-protocol-assistance#parallel-consultations-from-regulators-and-hta-bodies-section)	
	Review of public documents (e.g. Public Summaries of Opinion on orphan designations https://www.ema.europa.eu/en/medicines/ema_group_types/ema_orphan)	
	Patients as members of the EMA Scientific Committees: Committee for Orphan Medicinal Products (COMP) (https://www.ema.europa.eu/en/committees/committee-orphan-medicinal-products-comp), Committee for Advanced Therapies (CAT) (https://www.ema.europa.eu/en/committees/committee-advanced-therapies-cat), and Paediatric Committee (PDCO) (https://www.ema.europa.eu/en/committees/paediatric-committee-pdco)	
	Patients can also be consulted in writing or invited to attend committees in person (e.g. disease-specific requests)	
	Patients can be consulted in writing or invited to attend committees in person (e.g. disease-specific requests)	
Evaluation	Scientific advisory group (SAG) (https://www.ema.europa.eu/en/human-regulatory/research-development/scientific-advice-protocol-assistance)	Yes
	Patients can be consulted in writing or invited to attend Committees in person (e.g. disease-specific requests, benefit/risk assessment discussions at the Committee for Medicinal Products for Human Use https://www.ema.europa.eu/en/committees/committee-medicinal-products-human-use-chmp), and COMP for maintenance of orphan designation	
	Review of public documents, such as the Medicines Overviews, Herbal summaries and Package Leaflets, in order to review if the content is comprehensive and written in lay language	

[2] Patient Involvement along the medicine lifecycle at EMA. Available at: https://www.ema.europa.eu/en/partners-networks/patients-consumers/getting-involved

TABLE 2. **Types of EMA engagement activities[2] and DOI requirement**

Phase of the medicine lifecycle	Types of activities	DOI required
Post authorisation	Scientific advisory group (SAG) or ad-hoc expert meetings convened by the Pharmacovigilance Risk Assessment Committee (PRAC) (https://www.ema.europa.eu/en/committees/pharmacovigilance-risk-assessment-committee-prac). These are meetings where experts are invited to discuss specific scientific questions to inform the Scientific Committees' decision-making process	Yes
	Patients can be consulted in writing or invited to attend Committees in person (e.g. disease-specific requests, PRAC referrals, etc.)	
	Review of public documents, such as the safety communications, in order to review if the content is comprehensive and written in lay language.	
	Public hearings (https://www.ema.europa.eu/en/about-us/how-we-work/publichearings)	No

TABLE 3. **Types of patient input at EUnetHTA[3]**

Approach	Patient and/ or patient representative	Description of patient contribution & deliverables	Patient investment and compensation	Conflict of interest and confidentiality
Open call for patient input	Patient organisations (representatives) Patient will have access to information publicly available, no additional confidential data will be shared	General feedback from an organizational level; summarized view intended to be representative	Some investment for organizations; no compensation	Organizations provide information regarding funding and conflict of interest

[2] Patient Involvement along the medicine lifecycle at EMA. Available at: https://www.ema.europa.eu/en/partners-networks/patients-consumers/getting-involved

[3] Adapted from EUnetHTA Patient Input in Relative Effectiveness Assessments. Available at: https://eunethta.eu/wp-content/uploads/2019/06/Final_290519_Patient-Input-in-REAs.pdf. Updated 29.05.2019. Last accessed on 30 October 2019

TABLE 3. **Types of patient input at EUnetHTA[3]**

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

[3] Adapted from EUnetHTA Patient Input in Relative Effectiveness Assessments. Available at: https://eunethta.eu/wp-content/uploads/2019/06/Final_290519_Patient-Input-in-REAs.pdf. Updated 29.05.2019. Last accessed on 30 October 2019

TABLE 4. **Types of engagement instruments**[4]	
Focus group	A qualitative group discussion between participants, which is typically conducted in-person and moderated by a moderator with special expertise. Key considerations for focus groups may include research objectives, topic complexity, topic sensitivity, group size/number of participants per group, group diversity/heterogeneity, and number of focus groups needed.
Qualitative interviews	A semi-structured conversation between a subject and interviewer to collect rich, in-depth qualitative data related to the research question. This may include discussion about a patient's disease experiences, or their attitudes/perceptions towards treatment and its impact.
Online patient surveys	Completed by patients and can be used to collect quantitative data (discrete choices) or qualitative data (free-text answer boxes). Patients complete the survey independently, and do not interact with one another. Patient surveys are most valuable to assess stakeholder attitudes/perceptions and quantify disease burden/unmet needs; such surveys methods are indispensable when large datasets are required for quantitative analysis or when patient populations are geographically diverse.
Delphi panels	Multistage survey process with the intent to achieve consensus among experts, including patients, on a topic or issue. It can provide valuable data to help describe a patient experience or event[34]
Patient advisory panels/boards	Consists of patient experts (patients, patient advocates and/or caregivers) who are convened to bring unique knowledge to inform teams regarding the patient perspective. This tool places patients as strategic advisors rather than research participants.
Community advisory board (CAB)	Group of patients and patient representatives that serves as a link between a community and researchers/developers. Within clinical development, a CAB may review clinical trial protocols, monitor clinical trials, and help inform the community about them[35].
Public meetings	Offers the opportunity to tailor a meeting with patients, patient advocates, and a broader representative panel of decision-makers including clinicians, scientists, health authorities, and payers, to address specific objectives

[4] Types of patient engagement instruments table adapted from PARADIGM Recommendations on How to find the right match for the right patient engagement activity. Available at: http://imi-paradigm.eu/PEtoolbox/identification-of-patient-representatives