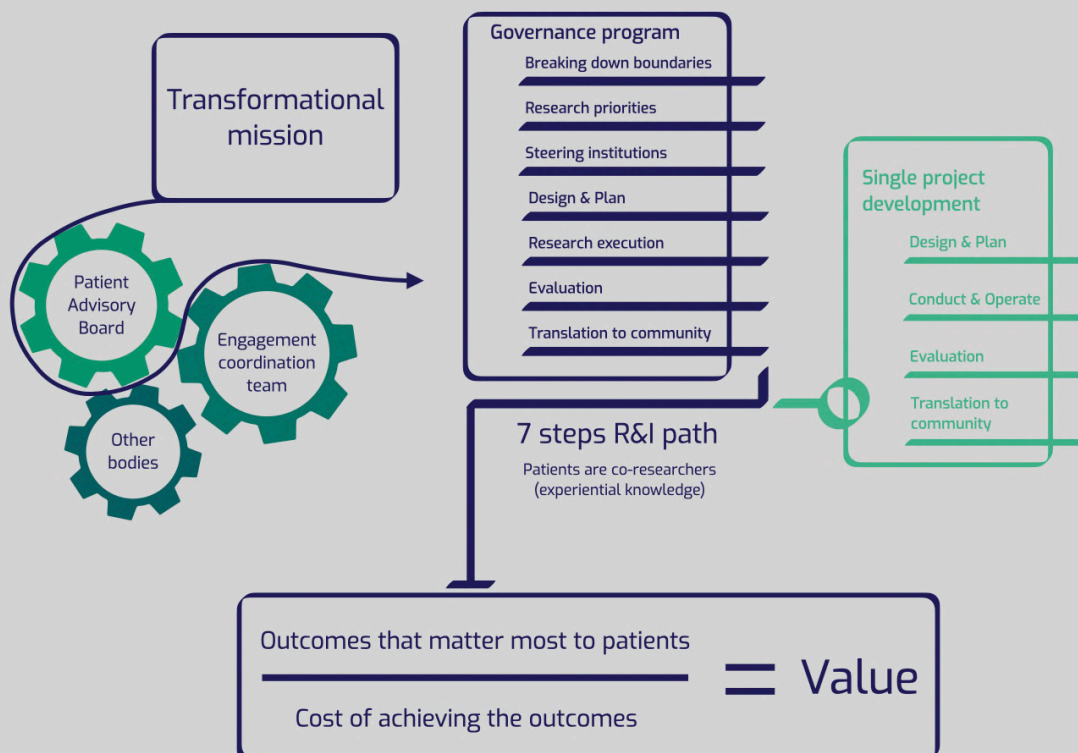


Guidelines for Patient Engagement in Health Research & Innovation

Short version

Version 0.1 - World Multiple Sclerosis Day 2020, May 30th



Introduction

MULTI-ACT is a three-year project funded within the H2020 Science with and for Society (May 2018 – April 2021)¹. The aim is to increase the impact of health research on people with brain disorders by providing a framework and tools for multi-stakeholder health research initiatives, allowing an effective cooperation of all the relevant stakeholders and the alignment of the results to the mission and agenda.

Over the last decade, patient engagement has become more important along with the democratization of health sciences. Patients started to be engaged not only in a passive role, but also as co-researchers. In fact, they can share with other stakeholders their own experience of the disease, which translates into a form of knowledge that integrates with scientific knowledge (experiential knowledge).

MULTI-ACT is developing a strategic *Collective Research Governance and Sustainability Model* in the area of brain diseases by using Multiple Sclerosis as the first case study. The project foresees patients² as a key stakeholder in the Health Research & Innovation (R&I) process. Hence, the project aims to contribute to the development of the “science with/of patient inputs” by providing guidelines for patient engagement in R&I (*with*) and by applying the patient-reported dimension in addition to the four impact assessment dimensions³ of the MULTI-ACT model (*of*). The purpose of the guidelines presented in this document is to provide multi-stakeholder research initiatives, promoted by Health Research Funding & Performing Organizations, with a tool to enable patient engagement actions into the R&I path at both program and project levels. These guidelines indicate how to apply the project’s patient engagement strategy within the activities of multi-stakeholder initiatives.

The guidelines propose a roadmap to capture ‘experiential knowledge’ of patients (i.e. knowledge gained through lived experience that researchers may not have), to better understand how to draw on their experience and use the experience constructively for co-creation purposes. MULTI-ACT aims at leveraging both patients and other stakeholders’ experience and at raising their ability to co-create and participate in decision making processes.

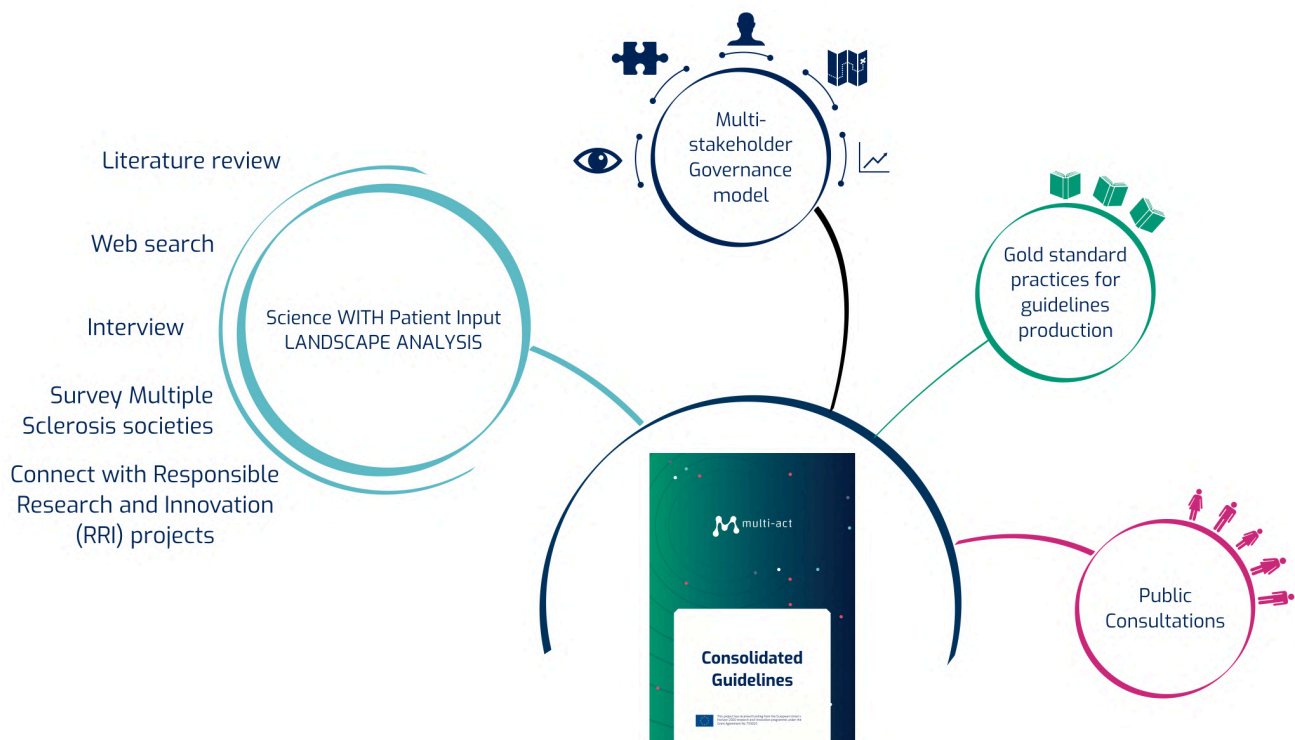
¹ <https://www.multiact.eu/>

² In order to clarify terminology for potential roles of patients’ interaction presented in this and other MULTI-ACT documents, we use the term “patient” which covers the following definitions:






- “People with the disease” are persons with lived experience of the disease;
- “People affected by the disease” are persons or groups that are affected by the disease, including family members and caregivers.

³ MULTI-ACT impact assessment dimensions: Mission/Efficacy, Excellence, Efficiency, Social and Patient Reported

The guidelines have been produced following a co-creation and evidence-based process which entails several steps. The guidelines include translation of the lessons learnt from the landscape analysis of existing patient engagement procedures (literature review, web-search, interviews, surveys, connections)⁴, resulting into guidance, recommendations, methods and suggestions on how to fill gaps identified with the landscape analysis. In line with existing good practice on guidelines production⁵, the guidelines were validated with a series of actions including a public consultation and review by key stakeholders.



The guidelines will be integrated into the MULTI-ACT Collective Research Impact Framework (CRIF) and finally downloadable via the MULTI-ACT Digital Toolbox. According to this framework, a multi-stakeholder initiative applying the MULTI-ACT Governance Model should be consistent with the following five criteria:

-  Define vision, scope and a shared agenda, considering MULTI-ACT principles;
-  Guarantee an inclusive and equitable governance model, which allows the involvement of all interested parties under a co-creation approach;
-  Guarantee a comprehensive, balanced and efficient stakeholder engagement process, ensuring the participation of patients, their families and caregivers;
-  Guarantee an effective, cooperative and efficient coordination and alignment of the objectives and actions required to pursue the vision and the agenda;
-  Establish a shared and effective assessment system, including a set of indicators, that allows co-accountability/co-responsibility of all the stakeholders.

⁴ D1.4 Consolidated mapping of existing patient engagement initiatives and analysis of gaps and barriers to patient. engagement in current health R&I processes (<https://www.multiact.eu/project-deliverables/>).

⁵ World Health Organization (WHO) handbook for guidelines production.

Guidelines

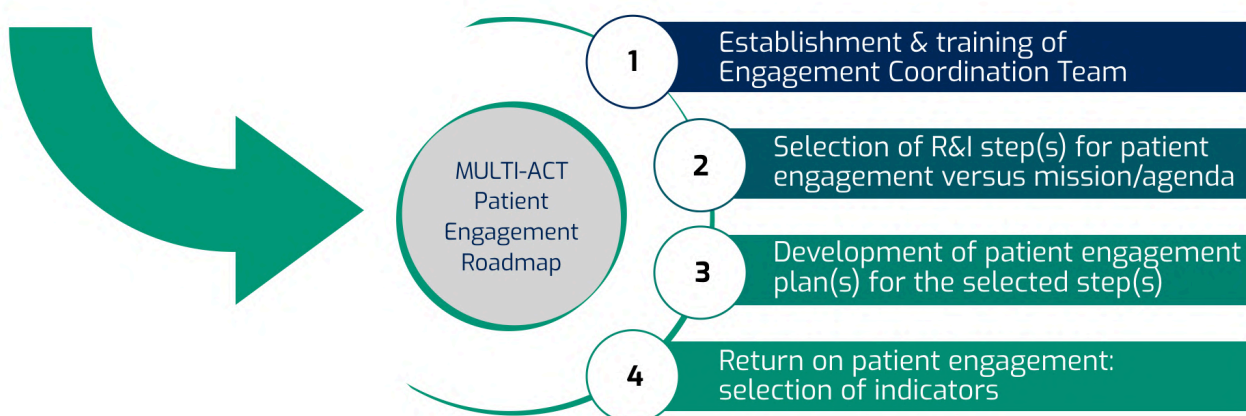
The MULTI-ACT Patient Engagement Guidelines provide a roadmap to enable patient engagement actions within the multi-stakeholder research initiatives that apply MULTI-ACT Governance Model and related criteria.

The MULTI-ACT Patient Engagement Roadmap consists of four fundamental activities: setting up an Engagement Coordination Team with trained figures; selecting the research steps where patients engagement is instrumental to meet the project's mission/agenda; developing an engagement plan for each research identified steps; and selecting the indicators to be used to measure the success and effectiveness of this engagement. Initiatives applying to use these guidelines should first perform a self-assessment check using the MULTI-ACT Digital Toolbox to verify whether they are coherent with the proposed governance model and criteria, including the patient engagement strategy.

MULTI-ACT Governance Model

A. Assessment of the Governance Model, included the Patient Engagement Strategy

B. Development of Governance Implementation Plan



The Engagement Coordination Team

The establishment of a board such as the Engagement Coordination Team is a pre-requisite for an effective use of the present guidelines. This team is in charge of the management of stakeholder engagement, including the design of the patient engagement strategy in order to empower the experiential knowledge of patients. Patients are key stakeholders of the Engagement Coordination Team, ensuring representativeness of their community.

Functions of the Engagement Coordination Team

The Engagement Coordination Team is a neutral body responsible for designing and actuating the Patient Engagement Plan, in charge of:

- ◆ creating commitment among the members and their community;
- ◆ moderating the dialogue between interdisciplinary and different (and sometimes competing) voices and experiences and settling a dispute resolution system;

- ◆ mitigating challenges such as ethical conflicts in protocol design, tokenism, power struggles, difficulties in recruiting different patients, additional time, cost;
- ◆ assuring that patients “feels valued” by facilitating team interaction and setting up an inclusive research environment.

Composition of the team

The composition of this team depends on the specificity of individual programs and/or projects. However, it is considered that the essential figures of this team are as follows:

- ◆ Co-Chair, patient (1);
- ◆ Co-Chair, MULTI-ACT trained representative⁶ (1);
- ◆ Initiative’s board(s) representative (1);
- ◆ Initiative’s staff representative (1);
- ◆ Patients (with consideration to balance of gender, geography, disease progression, age, socio-economic background) (3-6);
- ◆ Expert(s) on the mission/priorities topic(s): e.g. Working Group chair/representative.

The promoters of the multi-stakeholder initiative are in charge of establishing the Engagement Coordination Team and appointing or recruiting members. The appointment/recruitment should be compliant with the MULTI-ACT Governance and Patient Engagement criteria, assuring that the selection bias is mitigated and the mechanism is democratic. Patients and stakeholders’ selection can be achieved by a dedicated open “call for expression of interest”. A MULTI-ACT Patient Recruitment Plan relevant to the target mission will be developed, as for the other stakeholders, as part of the Patient Engagement Roadmap⁷. On the other hand, MULTI-ACT will exploit a training module for all the stakeholders involved to support the use of the MULTI-ACT Governance Model in the management of multi-stakeholder research initiative, including patient engagement and this training will form relevant candidates.

Main skills

The Team is an experimental new board that requires innovative training and educational programs that could also provide new job opportunities and careers. Some aspects to be considered, without being exhaustive, are motivational and coaching abilities and abilities in moderating and facilitating the management of diverse engagement methods (online and offline). The essential skills required to enable effective patient engagement are:

- ◆ Empathy and active listening compassion;
- ◆ Communication skills;
- ◆ Expertise in engagement strategies & methods (online and offline)⁸.

⁶ A training module to support the use of MULTI-ACT model in the management of multi-stakeholder research initiatives and patient engagement will be developed. The MULTI-ACT representative will be an individual that has successfully completed the training.

⁷ An ad hoc template to be customized for each mission and related Patient Engagement Plan(s) will be developed as practical tool to facilitate the application the MULTI-ACT Patient Engagement Roadmap (e.g. the “MULTI-ACT Patient Engagement call for interest template”).

⁸ Other preferred abilities are the following: Scientific knowledge of the disease; Personal experience of the disease as a patient, family member or caregiver; Team working abilities; Motivational and coaching abilities; Socio-psychological knowledge/background; Ethical management knowledge/background; Understanding of group dynamics; Project management knowledge/background.

Training features

Engagement Coordination Team members should receive adequate information about the programs and/or project's mission and strategy. An explanation of what is expected from patients and other stakeholders, of what are the expected outcomes of the multi-stakeholder initiative and how do those outcomes relate to the patients' needs in the given disease area should also be include in the training modules. The training should provide basic knowledge about innovative communication, learning and co-working techniques, and provide evidence of the value of patient and stakeholder engagement. In addition, plain language should be used and the content should be kept simple and respect for the human rights and the dignity of the patient should always be considered ⁹.

Terms of reference for the Engagement Coordination Team

- ◆ design and put in place actions to engage patients in the steps of the initiative/project and constantly monitor and review the patient engagement performance and Return on Engagement;
- ◆ direct and moderate the patient's experiential knowledge so that it is transformed into outcomes that matter to patients and are "scientifically" validated¹⁰;
- ◆ translate into a lay language eventual technical information that patients need in order to provide their feedback;
- ◆ motivate patients to stay engaged along the initiative/project by applying indicators to measure their Return on Engagement;
- ◆ assure the sustainability of the Patient Engagement Plan, monitoring risks and proposing mitigation actions.

The process for patient engagement in the 7-steps R&I path

The second action recommended by the roadmap (managed by the Engagement Coordination Team) is to define which are the steps of R&I path where patient engagement is instrumental to achieve the mission and agenda of the initiative.



Although patient engagement is considered crucial at every step of the research, the public consultation carried out by MULTI-ACT has prioritized these steps according to the relevance of the patients' role. This prioritization reflects the views given by the consulted stakeholders, which are partly based on the current state of patient engagement, and may not adequately valorize steps of research where patients and other stakeholders are currently underrepresented. The MULTI-ACT Governance Model aims to help mitigate this aspect.

⁹ MULTI-ACT is developing a training module (MULTI-ACT Training module®) to on how to gather patients' story and information and translate them into experiential knowledge useful in the context of R&I.

¹⁰ The Patient Reported Outcomes (PROs) are examples of "scientifically" validated measures to evaluate the impact of research on outcomes that matter to patients.

The prioritization is presented below:

1. Translation to community
2. Setting research priorities
3. Breaking down boundaries between patients and stakeholders
4. Research design and plan
5. Research evaluation
6. Steering institutions
7. Research execution

The list of actions associated with the R&I steps, classified by relevance according to the public consultation, is given in the Table 1 Menu of Patient Engagement activities along the 7-steps R&I path.

Table 1 Menu of Patient Engagement activities along the 7-steps R&I path.

7-steps R&I path	MENU of Patient Engagement Activities
BREAKING DOWN BOUNDARIES	<ul style="list-style-type: none"> • Patients help to define what are the boundary condition for patient engagement in multi-stakeholders initiatives • Patients help to provide an overview on the facilities and infrastructures they need to be engaged in R&I • Patients help to review the policies and guidelines on how to engage patients in R&I activities
RESEARCH PRIORITIES	<ul style="list-style-type: none"> • Patients are engaged to co-design research agenda. • Patients are engaged in advancing their interests in a specific research domain. • Patients are engaged to exercise prioritization.
STEERING INSTITUTIONS	<ul style="list-style-type: none"> • Patients are invited to be a member of committees and boards to provide guidance on key issues such as a company's policy and objectives, budgetary control, marketing strategy, resource allocation, and decisions involving large expenditures • Patients are invited to advise steering and advisory committees. • Patients are engaged in defining ethical issues, anticipating risks and barriers for patient engagement in committee.
DESIGN & PLAN	<ul style="list-style-type: none"> • Patients are engaged to suggest endpoints and outcomes of research. • Patients are engaged to propose specific objectives of research. • Patients are engaged to define the relevance and acceptability of proposed research to patient community.
RESEARCH EXECUTION	<ul style="list-style-type: none"> • Patients are engaged in the development and monitoring of research at Program Level (e.g. release of call for proposals, selection of projects to be funded, monitoring of funded projects) • Patients are engaged in the development and monitoring of research at Project Level (e.g. collaborating for ICT device development, for the enrolment to increase participation and decrease drop-down, to increase compliance with protocols and facilitate data collection, for writing and review of papers)

EVALUATION	<ul style="list-style-type: none"> • Patients are engaged in discussing in the multi-stakeholder team about new methods to measure the impact of research and align results to the mission/agenda. • Patients are engaged in the assessment of new approaches and products arising from research. • Patients are engaged in working with other stakeholders on research reports.
TRANSLATION TO COMMUNITY	<ul style="list-style-type: none"> • Patients are engaged in in shaping the “translation strategy” of research results to easy-to-use and easy-to-understand (lay) material and in communication activities to disseminate the research results • Patients are engaged in the development of guidelines and advocacy activities. • Patients are engaged in advocacy to leverage uptake of research results.

The Patient Engagement Plan

The third action recommended by the roadmap is to develop specific Patient Engagement Plans for the steps of the 7-steps R&I path that have been identified as relevant to meet the mission/agenda. The purpose of the Patient Engagement Plan is to provide a tool to integrate patient experiential knowledge in research, bringing to R&I expertise and knowledge complementary to the ones of the other stakeholders. Patients, as members of the Engagement Coordination Team and key stakeholders, develop the Patient Engagement Plan with the other stakeholders, ensuring representativeness of their community.

Minimum requirements of Patient Engagement Plan

The Patient Engagement Plan aims to be a managerial tool to support operationalization of patient engagement in research, and contain the minimum following requirements:

- ◆ Select actions of patient engagement that needs to be implemented in order to achieve the vision of the project;
- ◆ Define roles and responsibilities of the team that should manage and carry out the implementation of such patient engagement actions;
- ◆ Design methods to value and acknowledge the experiential knowledge of patients, including the establishment of appropriate recognition of patient contribution, and avoid tokenism;
- ◆ Choose clear and measurable targets (measuring the performance and Return on Engagement);
- ◆ Present a clear timeline of activities and sustainable budget;
- ◆ Define a clear review process (e.g. report on the performance and Return on Engagement);

Process to design the Patient Engagement Plan

The design of the Patient Engagement Plan relies on the following steps¹¹:

1. Define the purpose of patient engagement according to a given mission
 - Considering the given mission, how can patients help to meet the challenges ('utility in context')?
 - Which priorities of the mission/agenda would benefit more from patient engagement activities?

¹¹ In the appendix of these guidelines you can find a useful template to design an appropriate Patient Engagement Plan.

2. Define patient engagement expectations and objectives in relation to the 7-steps R&I path. This step must answer the following question¹²:
 - In which of the 7-steps R&I path do we need to engage patients?
 - What do we expect from patients?
 - What are the specific patient engagement actions for each of the step identified as relevant?
 - What type of patients do we need to engage? Identification of the profile of patients that should be engaged.
 - What discussion questions can be used to capture patients' experiential knowledge?
3. Define potential risks and mitigation plans.
4. Define measurements to assess the performance and the Return on Engagement.
5. Define clear and effective training of patients and researchers, to prepare patients for their engagement and in particular for taking part in conversations with researchers that support mutual learning (in line with the role of the Engagement Coordination Team)¹³.
6. Design a system of recognition that duly acknowledges patients' contribution in research, and the value of collaboration (*e.g. expenses reimbursed, results co-authored by patients, patients in peer-reviewers and open access research*).
7. Define budget and timeline for the execution of the Patient Engagement Plan.
8. Define reporting, meetings and communication channels. The reporting should include mechanisms to monitor and evaluate the performance and value of patient engagement ex-post and during implementation (*e.g. describe the review process in relation to the performance and value of the patient engagement, describe how the objectives for patient engagement are met, the performance and Return on Engagement, define the value for patient engagement: Patient Engagement Plan / Cost to put in place the Plan = Value¹⁴*).
9. Check that the plan complies with ethical regulations.
10. Check the coherence of the plan with the MULTI-ACT model and minimum requirements.
11. Define technicalities and operational aspects to enable a supportive research ecosystem.

Method to engage patients

This chapter presents the recommendations that seem to be the most appropriate in terms of methods of patient engagement¹⁵.

General recommendations for establishing the conditions for an effective partnership between the members of the Engagement Coordination Team

The selection process of patients and other stakeholders as partners should take into account communication skills, motivation, and constructive assertiveness in a team setting.

Patients and other stakeholders¹⁶ must be present from the beginning of the R&I process and considered on the same level as the other members and professionals of the Engagement Coordination Team, despite the differences in the contributions they can give (experiential knowledge versus scientific

¹² See the Appendix.

¹³ Training programs will be developed and validated by the experts appointed in the Engagement Coordination Team.

¹⁴ The reporting and monitoring system shall be defined on the basis of a shared vision (see D5.4 - criterion 5 - <https://www.multiact.eu/project-deliverables/>).

¹⁵ The evidence to support these recommendations comes from the landscape analysis carried out in D1.4, the preliminary guidelines drawn up by the consortium in D1.5, the public consultation and the literature review.

¹⁶ See glossary: Patients are people with the diseases and affected by the diseases (i.e. relatives, caregivers). Other stakeholders are Patients organizations, Society, Payers and purchasers, Care providers, Policy makers, Industry companies, Regulators, Research and education organizations Research Organizations

and technical expertise). This means that they want to "learn" but also to "teach" others in a co-learning perspective.

It is important to establish a supportive research ecosystem leveraging patient engagement (communication channels, resources, infrastructures, organizational/institutional). It is necessary to assure that patients understand and agree on the research agendas; and assure that they feel comfortable and recognize their unique perspective (motivation). **It is important** to empower team attitudes by creating an environment that support partnership, cultivating supporting attitudes in recognizing the partnership and keeping open dialogue (strong communication actions, shared goals). In order to allow full participation, the patients should be placed in the best possible conditions with regard to the needs of their condition. Respect and consideration must be guaranteed, so as to manage prejudice and hostile behaviour towards the participation of patients.

Patients should be guaranteed adequate empowerment to play their role in the team, first of all through comprehensible information, in their familiar language. There also should be adequate compensation in terms of Return on Engagement¹⁷, also aligned, as for the other stakeholders, to the expectations for their engagement and what they hope to achieve.

The best way to engage patients is to use mixed methods: *offline* methods (engagement without using computers, smartphones, tablets, or other internet-connected device/digital systems) and *online* methods (engagement through computers, smart phones, tablets, or other internet-connected device/digital systems)¹⁸. Online methods allow stakeholders to gather patient's perspective on a global scale while offline methods are useful to facilitate patients in providing their experiential knowledge as they may feel more comfortable to express their feelings face-to-face and they may be supported by a professional skilled managerial team (i.e. the Engagement Coordination Team). Moreover, offline methods allow stakeholders to discuss more in-depth and to establish and to maintain a good partnership with patients.

In the Engagement Coordination Team's work process, it is useful to increase public awareness and provide open access publication and return to patients' community. Prior to release of research outcomes, a final discussion with patients should assure consensual decision.

A partnership with culturally specific groups to ensure compliance among different cultures and geographical settings (especially underserved populations) should be sought. Cultural context, belief and tradition impact on patients' lived experience and provide additional insights to inform knowledge. Multimedia information technologies and tools are useful for monitoring conversations, proactively resolving complaints, and facilitating transparency, contributing to enhanced patient experiences and helping organizations comply with meaningful use criteria, such as engaging patients and families in their care, improving quality and care coordination, and reducing disparities. Implementation of social media may affect patient engagement and satisfaction in healthcare and research. The integration of social media into clinical practice and research can empower surgeons to synthesize effectively a patient support community that augments patient engagement and satisfaction. The same may apply as well to the R&I domain and environment.

Social media might have a determinant role in identifying patient insights and engage them in R&I for capturing their experiential knowledge. Evidence related to the efficacy and effectiveness of social media is currently limited. Various challenges have also been identified relating to privacy and security concerns, usability, the manipulation of identity, and misinformation. A great deal of momentum

¹⁷ Smith, E. et al. 2019. Patients as Research Partners: How to Value their Perceptions, Contribution and Labor? Citizen Science: Theory and Practice, 4(1): 15. pp. 1-13. DOI: <https://doi.org/10.5334/cstp.184>

¹⁸ Consolidated mapping of existing patient engagement initiatives and analysis of gaps and barriers to patient engagement in current health R&I processes (MULTI-ACT Deliverable D1.4).

surrounds the application of new technologies, such as mobile devices and other digital platforms, to both deliver care and generate real-world data on patients' experiences. The use of social media for patient engagement in research should be scientifically validated. The Science of Social Media as a method to capture patients' voice should be investigated.

Review of methods for the engagement of patients and other stakeholders

Following the results of the landscape analysis, the literature review, the public consultation, and the review of the members of the MULTI-ACT Patient Engagement Group and Patient Forum, historically used methods for patient engagement are the following: Focus Group, Democs Cards Games, World Café, Consensus Conference, Community Advisory Board, Delphi Method, Citizens Hearing, Serious Gaming, Research Studios methods, Scenario Workshops, World Wide Views, Voting Conference, Informed and deliberative surveys.

MULTI-ACT will exploit a dedicated training that will also build on historical methods that have features that might be relevant to capture experiential knowledge and Give Voice to Patients¹⁹, such as Focus Group and Democs card game.

The **Focus Group** is a qualitative method which is used to determine the preferences of people or to evaluate strategies and concepts. The method has originally been designed for market research. Participants are selected according to certain characteristics in common that relate to the research topic and are grouped into 8-10 people. It can be conducted face to face or in virtual digital space. The method is often used to generate or evaluate hypotheses and ideas in conjunction with a quantitative method, or as a primary data-collection method. Focus group is undoubtedly the most widespread technique of engagement. It is rooted in qualitative studies, where it is a standard way of gathering patients' input and learning about their views and experiences. Its scope of application has widened in recent years, with the method being used for decision-making and guidelines formulation, not without some criticism regarding insufficient separation of these two functions.

Example: Selected patients and stakeholders are invited to a meeting to discuss about a topic.

The **Democs** is both a card game and a policy-exploration tool that enables small groups of people to engage with complex public policy issues. It aims to help people find out about a topic, express their views, seek common ground with the other participants, and state their preferred policy position. There are already a number of Democs kits on different issues which can be bought or downloaded for free from New Economics Foundation (NEF) and Play Decide (<https://playdecide.eu>).

Example: Patients are provided with discussion cards that help them to express their views on a topic, to seek common ground with the other participants, and to express their preferences.

Measure the performance and effectiveness of patient engagement

The last part of the guidelines presents a selection of indicators that can be used for assessing the Return on Engagement, to be monitored and used at the end of the engagement to evaluate if it has reached the expected impact on the initiatives²⁰. In particular, we provide here a list of metrics to evaluate:

¹⁹ Boenink M, van der Scheer L, Garcia E, van der Burg S. Giving Voice to Patients: Developing a Discussion Method to Involve Patients in Translational Research Nanoethics. 2018;12(3):181-197. doi: 10.1007/s11569-018-0319-8. Epub 2018 Jul 9.

²⁰ This action is linked to the overall assessment detailed in Work Package n. 3 Deliverable D3.6 "Master Scorecard" and will be further detailed in Deliverable D1.8 "Patient Reported Dimension Report on the integration of Patient Reported Outcomes and perspective into the Collective Research Impact Framework (CRIF)".

- ◆ the *performance* of patient engagement (i.e. the success of the initiative in terms of participation).
- ◆ the *effectiveness* of patient engagement (i.e. the success of the initiative in term of real impact of the participation on the research process)²¹.

Performance Assessment

To measure the performance (success) of the engagement process, the core metrics that emerged from our research are as follows (classified according to the preference given by the participants in the public consultation):

- ◆ The number of different phases of the research process patients have been engaged in.
- ◆ The number of patients engaged across different socio-economic statuses, education backgrounds, genders, etc., to assess the capacity to engage diverse groups, including the most vulnerable ones.
- ◆ The number of engagement actions (online and offline) that have taken places in which patients have had the possibility to express their views.

Effectiveness and value assessment

To measure the value and effectiveness of the engagement process, some metrics emerged as more reliable²²:

- ◆ The number of changes in the research process (e.g. policies, composition of boards, objectives and priorities, strategic plan, evaluation of results, dissemination actions, etc.) according to the review made by patients.
- ◆ The number of research initiatives, programs and projects that include and show an effect on Patient Reported Outcomes (i.e. questionnaire reporting how they feel about symptoms and functions).
- ◆ The number of research initiatives, programs and projects involving patients in research activities, according to the needs of the mission.

Qualitative:

- ◆ The analysis of whether patients' expectation with respect to the research and mission of the initiative are met.
- ◆ The analysis of the achievement in terms of new knowledge produced, from the perspective of all the stakeholders.

²¹ The metrics to evaluate the effectiveness of patient engagement will be further developed under the activities of Task T1.5 and results will be included in Deliverable D1.8 "Report on the integration of Patient Reported Outcomes and perspective into the CRIF". These metrics will be included in the Multi-Act Digital Toolbox as a sub-set of the Patient Reported Dimension.

²² Metrics have been ranked according to the preference given by participants at the public consultation.

Conclusions

The comprehensive instructions on how to apply the MULTI-ACT framework in all its parts (Governance Model, the Patient Engagement Strategy, and the Impact Assessment) will be released in the MULTI-ACT Manual²³ and in the Digital Toolbox²⁴. Relevant future activities will be directed to the exploitation of these guidelines. In particular a training module to support the use of MULTI-ACT model in the management multi-stakeholder research initiatives and patient engagement will be developed²⁵. Within this module special attention will be directed to patients and stakeholders' training on how to capture patient experiential knowledge.



²³ Deliverables of Work Package n.6 Collective Research Politics: governance and guidelines of MULTI-ACT project.

²⁴ Deliverables of Work Package n.2 Development of the information sharing application (MULTI-ACT Toolbox 2.0) of MULTI-ACT project.

²⁵ MULTI-ACT Academy on Multi-Stakeholder Research Initiatives and Patient Engagement management.

Glossary

Collective Research Impact Framework (CRIF): conceptual framework that MULTI-ACT is developing to enable a new collective accountability approach to multi-stakeholder R&I initiatives in the field of brain diseases.

Engagement Coordination Team (referred to as “ECT”): the board that is in charge of coordinating the engagement of stakeholders, including patients, relatives and caregivers, in all the operations of an initiative (see deliverable D5.4 MULTI-ACT Governance Model);

Experiential knowledge: “Experiential knowledge arise when [these] experiences are converted, consciously or unconsciously, into a personal insight that enables a patient to cope with individual illness and disability. When patients share experiential knowledge, the communal body of knowledge exceeds the boundaries of individual experiences.”²⁶ Thus, experiential knowledge is knowledge gained through experience, as opposed to a priori (before experience) knowledge.

Patient engagement in Responsible Research and Innovation (RRI): actions to engage patients in R&I processes to make them co-responsible (as sub-group of stakeholders). In line with RRI definition, patient engagement implies that patients work together with other stakeholders (researchers, citizens, policy makers, business, third sector organisations, etc.) in order to align both the process and its outcomes with their values, needs and expectations. The role of patient in research ranges from a passive one (patient is a data point), to an active one (patient is a researcher) and finally to a strategic one (patient has an active role in the governance and decision making of research process) – see: levels of engagement.

For the purpose of MULTI-ACT, patient engagement is the action to engage patients and their communities in R&I as key stakeholders with a decision-making role, “occurring when people with and affected by the disease meaningfully and actively collaborate in the governance, priority setting, and conduct of research, as well as in summarizing, distributing, sharing, and applying its resulting knowledge”²⁷. While various contexts use different terminology to describe the engagement process, such as Patient and Public Involvement (PPI) in the UK²⁸, MULTI-ACT uses the term **patient engagement** and defines patient engagement as the action to engage patients in R&I processes to make them co-responsible for research (as sub-group of stakeholders: Science with patient input). Terms other than patient engagement (e.g. patient involvement) reported in this document refer to literature references or answers received during the public consultation.

Patient(s): In order to clarify terminology for potential roles of patients’ interaction presented in this and other MULTI-ACT documents, we use the term “patients” which covers the following definitions:

- **“People with the disease”:** persons with lived experience of the disease;
- **“People affected by the disease”:** persons or groups that are affected by the disease, including family members and caregivers.

Patient-Provided Information: a range of input or data that is collected from the patients²⁹.

Patients’ organizations: consumer advocacy organizations involved with the population of interest. “Patients’ organisations are defined as not-for profit organisations which are patient focused, and whereby patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies”³⁰. Within the context of MULTI-ACT Patients’ organizations play an important role in patient engagement as boundary body between priorities/outcomes that are individual patients’ perspective (a, b) to priorities/outcomes that work at population level. Patient Organization’s Representatives are persons who are mandated to represent and express the collective views of a patient organization on a specific issue or disease area.

Promoter(s): promoters are the actors that decide to implement MULTI-ACT Governance Model within their existing or new organizations. After the implementation of the Model, they will be part of the governance bodies (i.e. Leadership Board) (see D5.4).

²⁶ Caron-Flinterman, JF, Broerse, JEW and Bunders, JFG. 2005a. The experiential knowledge of patients: A new resource for biomedical research? *Social Science & Medicine*, 60(11): 2575–2584. DOI: <https://doi.org/10.1016/j.socscimed.2004.11.023>

²⁷ de Wit M, Abma T, Koelewijn-van Loon M, et al.: Involving patient research partners has a significant impact on outcomes research: a responsive evaluation of the international OMERACT conferences. *BMJ Open*. 2013; 3(5): pii: e002241.

²⁸ The term patient and public engagement has largely predominated in North America (<https://researchinvolvement.biomedcentral.com/articles/10.1186/s40900-019-0162-2>)

²⁹ https://www.nationalhealthcouncil.org/wp-content/uploads/2019/12/NHC_Patient_Engagement_Rubric.pdf

³⁰ <https://www.eu-patient.eu/About-EPPF/what-is-a-patient-organisation/>

Research & Innovation Path (R&I Path): sequence of processes and activities in R&I where patients can be engaged in order to maximize the impact of R&I. Governance Program Level and Project Development Levels are distinguished (also see: Governance Initiative):

- **Program Level:** Governance Program stages in multi-stakeholder initiative process concerned with the governance and management of research funding & performing programs:
 - **Breaking down the boundaries** conditions that should be set in Research Funding & Performing Organizations (RFPO) in order to facilitate patient engagement as standard practice.
 - **Setting research priorities** actions to establish justified interest in a specific research domain to a certain higher degree, importance, precedence, or rank over others.
 - **Steering institutions** actions performed to establish steering and advisory committees and bodies.
 - **Design and planning** the design and planning of all the activities that lead to the realization of a concept or idea and which helps achieve the item's designated objective(s).
 - **Executing research** activities to actualize the research program or a specific research project for the purpose of achieving the item's designated objectives. Project Development Level takes places at this stage.
 - **Evaluating research** activities to determine the value created by a research program or project, establishing their outputs and outcomes, the degree to which their pre-established goals were achieved, and their impact.
 - **Translation to community** activities to foster and facilitate the uptake of results of research programs or projects.
- **Project Level:** Project Development stages in multi-stakeholder initiative process concerned with performing single research projects (e.g. single project for the development of an ICT Health monitoring device or clinical trials). In this case, patient is a scientist and co-researcher. Project Development pertains to Research Execution stage of the Governance Program Level.
 - **Design & plan** the design and planning of all the activities that lead to the realization of a concept or idea and which helps achieve the item's designated objective(s).
 - **Conduct & operate** project conduct & monitoring (e.g. ICT device development).
 - **Evaluation** activities to determine the value created by a research project, establishing their outputs and outcomes, the degree to which their pre-established goals were achieved, and their impact.
 - **Translation to community** activities to foster and facilitate the uptake of results of research projects.

Return on Engagement (RoE): the benefit and impact resulting from performing patient engagement in R&I. Evaluating whether engagement adds value for different stakeholder groups can be an effective tool to further support patient engagement and requires the development metrics to measure the “return on engagement”. It should always be evaluated by both the engaging and engaged parties in line with the co-accountability approach of MULTI-ACT.

Return on Investment (ROI): a measure of the efficiency of an investment as a percentage of return relative to the investment's cost.

Science with patient input. intellectual and practical activity that occurs when patients meaningfully and actively collaborate in the governance, priority setting, and conduction of research, as well as in summarizing, distributing, sharing, and applying the results. In the context of MULTI-ACT, the Science with patient input aims to maximize the impact of R&I toward a transformational mission by engaging patients in R&I. The Science with patient input will be executed in the MULTI-ACT Governance model by applying the MULTI-ACT Patient Engagement Strategy included in the present guidelines.

Science of patient input: intellectual and practical activity that occurs when data of people with a disease are collected and used (active and passive contribution) to evaluate impact of R&I. In the context of MULTI-ACT, data about patients' experiences outside the clinic (Science of patient input) are critical to evaluate the impact of mission-oriented health research on outcomes that matter most to patients³¹. A great deal of momentum surrounds the application of new technologies, such as mobile devices and other digital platforms, to both deliver care and generate real-world data on patients' experiences.

Social media: forms of electronic communication (such as websites for social networking and microblogging) through which users create online communities to share information, ideas, personal messages, and other content (such as videos). In this document, for “Science of Social Media” we intend the use of social media and social networks to ensure representativeness of the different communities for science and research purposes. This use is subject to a scientific validation.

³¹ The Master Scorecard provides a selection of (qualitative and quantitative) indicators of research impact enable the translation of MULTI-ACT mission and agenda into action, integrating a set of top indicators on efficacy, efficiency, excellence, social impact and patient reported impact, co-selected within a multi-stakeholder perspective.

Stakeholder “any individual or group that is affected by, who can influence or may have an interest in the outcomes of an organization’s actions”.³²

- **Patients:** people with the disease (persons with lived experience of the disease); and people affected by the disease (persons or groups that are affected by the disease, including family members and caregivers).
- **Patient organizations:** patient associations, advocacy organizations, etc.
- **Society individual citizens,** civil society organizations and networks.
- **Payers and purchasers:** public or private entities responsible for underwriting the costs of health care.
- **Care providers:** health and social care organizations and professionals (doctors, nurses, etc.).
- **Policy makers:** EU institutions; national, regional and local policy makers.
- **Regulators:** regulatory agencies (e.g. agencies for the scientific evaluation and safety monitoring of medicines, i.e. the European Medicine Agency EMA); Health Technology Assessment (HTA) bodies.
- **Industry:** companies developing and selling health products (drugs, devices, applications, etc.) and services.
- **Research and education organizations:** Research Organizations; Universities; Education Providers; Foundations; Other research projects.
- **Transformational mission:** a mission as transformational or transformative means 'changing forms'. Transformational health research is a term that became increasingly common within the science and health policy community in the 2000s for research that shifts or breaks existing scientific paradigms.

³² R. Edward Freeman’s Strategic Management- A Stakeholder Approach. 1984.

Appendix

The template to support the design of the Patient Engagement Plan

The MULTI-ACT model offers a practical template to support the design of the Patient Engagement Plan. The indication included in the template aims to provide practical guidance to research and funding organizations on how to plan, actuate and monitor their patient engagement actions. The final version of the MULTI-ACT Digital Toolbox will provide tools to support the development of the Patient Engagement Plan. As far as common good practices can be outlined, each initiative will have unique and requires *ad-hoc* interventions.

Please note that fields with asterisks (*) in the Patient Engagement Plan are mandatory fields to enable patient engagement.

INITIATIVE/PROJECT TITLE:	
MISSION/SCOPE: <i>Briefly describe the mission and vision and its specific objectives in a language that is clear and understandable by multi-variate stakeholders.</i>	
1. PURPOSE OF PATIENT ENGAGEMENT* <i>Considering the mission, how can patients and stakeholders help to meet the challenge?</i>	
Patient engagement goals and challenges <i>List the goals and challenges</i>	How patients can help to meet the goals and overcome barriers <i>Describe how patients can help to meet the goals/overcome barriers.</i>
2. PATIENT ENGAGEMENT EXPECTATIONS IN RELATION TO THE 7-STEPS R&I PATH* <i>What we expect from patients? What type of patients we need to engage? What expertise we need to engage? What are discussion questions to capture patients' experiential knowledge? (Note: Given the expectation from Leadership Board, the Engagement Coordination Team (ECT) identify level of engagement, type of patients and requirements. An initiative/project does not necessarily have to act on all the steps)</i>	
BREAKING DOWN BOUNDARIES	Expectations: <i>Example - Patients help to identify requirements, roles and skills of boards in charge of patient engagement in order to integrate the patients' experiential knowledge into the R&I process.</i> Actions plan 1: <i>Example - Define a method to asks patients to provide an overview on the facilities, infrastructures, tools they need to be engaged in research.</i> Level of engagement: <i>Example - Co-design</i> Type of patients' representative: <i>Example - people with and affected by the disease, including family members and caregivers</i> Requirements: <i>Example - No specific or scientific expertise of patients is required other than their experiential knowledge</i>
RESEARCH PRIORITIES	Expectations: <i>Example - Patients help to identify and prioritize the unmet needs of patients</i> Actions plan 2: <i>Examples - Action 2.1: ETC and working group (WG) design and launch a "Public consultation" to identify patients' needs, relevance of initiative/project approach and confirm compliance with the initiative/project direction. (online method)</i> <i>Action 2.2: ECT organize a Focus group with WG (and other relevant stakeholders) to revise the initiative/project according to the outcomes of the public consultation. (offline method)</i> <i>Action 2.3: ECT and WG works remotely to integrate outcomes of Action 1.1 and Action 1.2 into the development of the initiative/project.</i> Level of engagement: <i>Example - Consult</i> Type of patients' representative: <i>People with and affected by the disease, including family members and caregivers</i> Requirements: <i>No specific or scientific expertise of patients is required other than their experiential knowledge</i>

STEERING INSTITUTIONS	<p>Expectations: <i>Example - Patients are enabled to integrate their experiential knowledge in R&I being part of the governance and having decision making power.</i></p> <p>Actions plan 3: <i>Example - Action 3.1: Establish governance bodies to enable patient engagement in line with MULTI-ACT Governance Model (i.e. ECT)</i></p> <p>Level of engagement: <i>Example - Co-design</i></p> <p>Type of patients' representative: <i>Patients, family members and caregivers</i></p> <p>Requirements: <i>No specific or scientific expertise of patients is required other than their experiential knowledge</i></p>
DESIGN & PLAN	<p>Expectations: <i>Example - Patients help to co-design specific programs/project</i></p> <p>Actions plan 4: <i>Example - Action 4.1: ECT engage patients as evaluators in the selection of funding or as peer-reviewers</i></p> <p>Level of engagement: <i>Example - To be defined based on the identified actions</i></p> <p>Type of patients' representative: <i>Example - To be defined based on the identified actions</i></p> <p>Requirements: <i>Example - To be defined based on the identified actions</i></p>
RESEARCH EXECUTION	<p>Expectations: <i>Example - Patients help the execution of R&I as co-researchers providing experiential knowledge.</i></p> <p>Actions plan 5: <i>Example - Action 5.1: ECT engage patients for helping in recruitment and data collection</i></p> <p>Level of engagement: <i>Example - To be defined based on the identified actions</i></p> <p>Type of patients' representative: <i>Example - To be defined based on the identified actions</i></p> <p>Requirements: <i>Example - To be defined based on the identified actions</i></p>
EVALUATION	<p>Expectations: <i>Example - Patients help the evaluation of R&I on the outcomes that matter most to them.</i></p> <p>Actions plan 6: <i>Action 6.1: ECT engage patients for data analysis and interpretation, patients asked to design PROs that matter to them.</i></p> <p>Level of engagement: <i>To be defined based on the identified actions</i></p> <p>Type of patients' representative: <i>To be defined based on the identified actions</i></p> <p>Requirements: <i>To be defined based on the identified actions</i></p>
TRANSLATION TO COMMUNITY	<p>Expectations: <i>Example - Patients endorse the R&I results and help their translation to community as ambassadors.</i></p> <p>Actions plan 7: <i>Example - Action 7.1: ETC engage patients in communication activities and outreach, patients co-authored publications and conduct knowledge translation.</i></p> <p>Level of engagement: <i>Example- To be defined based on the identified actions</i></p> <p>Type of patients' representative: <i>Example - To be defined based on the identified actions</i></p> <p>Requirements: <i>Example- To be defined based on the identified actions</i></p>
Wrap-up for all steps	<p>Considering all the action plans, summarize the actions, type of patients and requirements instrumental to define/implement governance boards composition (i.e. ECT and WGs).</p> <p><i>Note 1: define if it is enough a working group for all the steps or if there is the need of multiple working groups. working groups are coordinated by the ECT.</i></p> <p><i>Note 2: assure to be sustainable and to maintain an easy structure.</i></p>

3. RISKS AND MITIGATION PLAN

Risks	Mitigation plan
<i>Anticipate potential risks</i>	<i>Propose mitigation plan for the risk</i>
<i>Low participation of the patient community</i>	<i>Taking advantage of the network of patient organizations and relationships establishment</i>

4. PATIENT ENGAGEMENT PERFORMANCE ASSESSMENT*

MULTI-ACT provides a menu of indicators

Objectives	Means of verification
<i>Define objectives for evaluating the PE Plan</i>	<i>Clearly define how you are going to verify that the objectives are met.</i>
<i>Example: Patient engaged with mixed methods</i>	<i>Example: Number and type of methods used and events that have taken places to grant patients the possibility to express their views/experiences</i>
<i>Vision and Agenda meet the needs of patients</i>	<i>Number of reviews/changes made by patients to the Vision and Agenda according to the gaps identified by patients</i>
<i>Outcomes of R&I are co-developed and endorsed by patients</i>	<i>Number of reviews/changes of outcomes related to the 7-steps R&I path produced and endorsed by patients</i>

5. TRAINING FOR PATIENTS and ECT*
Describe the training program related to the Patient Engagement Plan

Please note that the MULTI-ACT Training module® for the ECT is under development to address the needed skills.
SCOPE:
Examples:
 Explain the mission and vision
 Provide basic information on the topic, the research context and process
 Explain what is expected from patients and the benefit of engagement (i.e. Patient Engagement Plan)
 Keep informed on progress (regularly update)
ACTIONS:
Example:
 Online/offline training sessions
 Online engagement methods duly anticipated by exhaustive Information Sheet

6. RECOGNITION AND REWARDS - VALUE OF COLLABORATION*
Clearly state the mutual benefit of engagement and the mechanism to assure it. Please note that the template presents the rewarding model of "Smith, E, et al. 2019. Patients as Research Partners; How to Value their Perceptions, Contribution and Labor? Citizen Science: Theory and Practice, 4(1): 15." as example. A general description of recognition mechanism is sufficient.

Financial	Compensation for expenses incurred when participating in research activities (e.g., travel, fuel, parking)
Personal	Thank-you letter Public mention and acknowledgment (e.g., in social events, on social media) Certificate of participation
Knowledge	Access to publications resulting from the research to which they contributed Access to training Access to scientific literature (or other types of knowledge) Opportunities to exchange with researchers and other PPRs after completion of the project
Academic	Acknowledgement in knowledge transfer communications Acknowledgement in articles Invitations as speakers at scientific conferences Co-authorship in articles
Altruistic	Moral satisfaction Augmentation of self-worth Augmenting wellbeing of others
Other	

7. PRELIMINARY BUDGET FOR THE PLAN*
Define the cost and person months (PM) for the actions resulting from this Plan

The 7-steps R&I path	Expected costs
BREAKING DOWN BOUNDARIES	Cost and PM for needed infrastructure set-up Cost and PM for ECT establishment and training
RESEARCH PRIORITIES	Cost of Public consultation Cost for Focus Group (if other representatives beyond ECT)
STEERING INSTITUTIONS	Cost and PM for the actions defined in step "Steering institution"...
DESIGN & PLAN	Cost and PM for the actions defined in step "Design & plan"...
RESEARCH EXECUTION	Cost and PM for the actions defined in step "Research execution"...
EVALUATION	Cost and PM for the actions defined in step "Evaluation"...
TRANSLATION TO COMMUNITY	Cost and PM for the actions defined in step "Translation to community"...

SUSTAINABILITY		
FUNDIGN SOURCES [Consider and define the funding sources to cover budget for Plan]	What percentage of budget may be dedicated to the actualization of Plan?	
8. REPORTING, MEETINGS & COMMUNICATION CHANNELS		
Channels	Use	
Meetings (Face to Face, virtual)	Meetings among the ECT	
emails	Formal and informal communication	
Reporting format	The Report is expected at year 1, year 2 describing: - the review process in relation to the performance and value of the patient engagement; - how the objectives for patient engagement are met (both on performance and on return on engagement), - the value for patient engagement (Plan outcomes/ Cost to put in place the Plan = Value).	
9. ETHICAL ASSESSMENT/ ETHICAL COMPLIANCE OF THE PLAN*		
Describe any ethical aspects to be considered in the plan and propose compensative actions in case of gaps		
Actions	Ethical aspects	Tools, mean of verification
Action 1.1: Establish PE governance bodies (i.e. ECT)	Agreement with the boards' members	Term of Reference for boards (i.e. ECT)
Action 2.1: "Public consultation": Data collection and management	Develop/check a Data Management Plan (DMP) and integration of patient perspective into its development	Informed sheet, Informed consent. Possibility to edit and review DMP with a simple sharing tool (e.g. google drive doc, etc.)
Action 2.2: Focus group with including people with high disability (e.g. in wheelchair)	Check accessibility of venue, agenda and timing not stressful. Possibility of web-streaming and recording in case the person cannot participate in person the day of the meeting.	Accessible location, light agenda. Recording and web-streaming of the meeting, possibility to give late contribution
Action 2.3: ECT works remotely to integrate outcomes	Compliance with respect of time	Appropriate technologies to connect and facilitate the people involved in activities
10. COMPLIANCE OF THE PLAN TO THE MULTI-ACT CRITERIA		
Check the criteria for patient engagement and list the criteria that are NOT met and if those may affect the performance or the value of the engagement.		
Check file MULTI-ACT PATIENT ENGAGEMENT CRITERIA – see D1.6 - Appendix 5		
11. TECHNICALITIES, OPERATIONAL ASPECTS		
List material and document to be prepared and other technicalities		
Timeline for the Plan (GANTT) – PLAN ANNEX 1		
Description of rationale for deciding methods to be used – PLAN ANNEX 2 (See D1.6 for suggested methods)		
Etc.		