

Public Consultation

Patient engagement

Results and key findings 2020

Introduction

The Consortium designed and launched a public consultation targeted to Health Research & Innovation (R&I). Through a web survey, the Consortium engaged stakeholders of Health R&I to share their views on specific aspects of the patient engagement in research and innovation (R&I) in order to consolidate the MULTI-ACT Patient Engagement Strategy with relevant insights.

Stakeholders have been invited to participate in the online consultation to express their opinions, perspectives and thoughts on how patient engagement should be implemented at different steps of the R&I process, in order to maximize its impact on the outcomes that matter most to people. By participating in the consultation, stakeholders contributed to the development of the MULTI-ACT Patient Engagement Strategy and guidelines, for making R&I more responsible toward patients and society.

The consultations represented a chance to hear from all concerned stakeholders in order to focus on where the Consortium needed to direct attention and research.

The survey was directed to all Health R&I stakeholders, categorized as follow:

- **Patients:** people with the diseases and affected by the diseases, including relatives and caregivers.
- **Patients organizations:** patient associations, advocacy organizations, etc.
- **Society:** individual citizens, civil society organizations and networks.
- **Payers and purchasers:** public and/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, including regulators.
- **Industry:** companies developing and selling health products (drugs, devices, applications, etc.) and services.
- **Research and education organizations:** Research Organizations; Universities; Education Providers; Foundations; research projects

A 7-weeks online public consultation was launched the 31st October 2019, accessible via the MULTI-ACT website, social networks, newsletters, and partner networks and distribution lists. The survey was online from 31st October 2019 to 30th November 2019. Considering the received interest and specific request for extension, the Consortium decided to extend the survey for 10 more days, until the 20th December 2019.

The questionnaire protocol includes a total of 24 questions. The survey comprised five demographics questions (i.e. name, age, country, gender, and stakeholder category), two general questions on previous experiences of patient engagement, fifteen questions on specific topics the Consortium wanted to consolidate with stakeholders, and two questions on closing remarks. All questions were optional for compliance to privacy and ethical regulations.



Questionnaire protocol

Code	Core	Question	Purpose	Topic
Q1	Core	Name and surname	Gather demographics of the study population	Demographic
Q2	Core	Age	Gather demographics of the study population	Demographic
Q3	Core	Country	Gather demographics of the study population	Demographic
Q4	Core	Gender	Gather demographics of the study population	Demographic
Q5	Core	Which group describes you best? (please choose the most relevant one)	Categorize the study population	Demographic
Q6	Core	Do you have some experience of patient engagement?	Assess if the respondents have experiences of patient engagement.	Background
Q7	Additional	If you wish, please give us more info about your experiences (e.g. which type of engagement)? Where and when did it happen? What was your role? Did you find the engagement useful for your research activities? etc.) (max 600 characters)	Assess the patient engagement's experiences of the respondents.	Background
Q8	Core	<p>The main task of the Engagement Coordination Team is to make it possible for patients to be genuinely engaged, to ensure representativeness of patients' contribution, to motivate them to participate, to bring out their experiences that are useful for research, to make the results of the research comprehensible to all and to stimulate feedback. Which of the following statements do you consider most appropriate for the purpose?</p> <p>Please choose the most relevant ones</p>	Gather approval and prioritization on the main assumptions with respect to patient engagement.	Assumptions for patient engagement
Q9	Core	<p>What is the most important benefit of engagement in scientific research and innovation for patients?</p> <p>Please choose the most relevant one</p>	Gather approval and prioritization on the proposed benefit of engagement in scientific research and innovation for patients, and identify additional benefit.	Benefit of engagement for patients
Q10	Core	<p>The patient's disease experience is of enormous value in directing research towards relevant goals. This experience is composed of many different aspects: needs, feelings, uncertainties, stories, reflections, insights, etc. The task of the Engagement Coordination Team is to translate these inputs into an experiential knowledge useful for research.</p> <p>What skills do the Engagement Coordination Team's members need most to enable patients make their experience valuable for research?</p> <p>Please choose the most relevant ones (up to three).</p>	Gather approval and prioritization on the proposed skills and abilities of the ECT and identify additional skills.	ECT skills and abilities
Q11	Core	<p>What is the most suitable training for the Engagement Coordination Team to integrate patient experiential knowledge in research?</p> <p>Please choose the most relevant ones (up to three).</p>	Gather approval and prioritize the proposed features for the ECT training modules, and identify additional features.	ECT training modules
Q12	Core	<p>What is the best way to measure the performance of patient engagement in research?</p> <p>Please choose the most relevant one.</p>	Gather approval and prioritization on the proposed metrics to assess the performance of patient engagement, and identify additional metrics.	Performance of patient engagement

Code	Core	Question	Purpose	Topic
Q13	Core	<p>In MULTI-ACT, patient engagement should cover every step of the research and innovation activities. We have identified 7 steps in which patients should be engaged with other stakeholders in this process, according to the mission and objectives of the program and/or project.</p> <p>At which step of the research process do you think patients should be engaged?</p> <p>Please choose the most relevant ones.</p>	Gather approval on the 7 steps R&I path as path where patients need to be engaged in order to maximize the impact of R&I, and implement the path with missing steps (if any), and/or prioritize the steps.	7 steps R&I path
Q14	Additional	If you selected 'Breaking down boundaries between patients and other stakeholders' in question 13, please choose the most appropriate patients' activities in this step.	Implement the step n.1 with examples of patient engagement activities	7 steps R&I path
Q15	Additional	If you selected "Setting research priorities" in question 13, please choose the most appropriate patient's activities in this step.	Implement the step n.2 with examples of patient engagement activities	7 steps R&I path
Q16	Additional	If you selected "Steering institutions" in question 13, please choose the most appropriate patients' activities in this step.	Implement the step n.3 with examples of patient engagement activities	7 steps R&I path
Q17	Additional	If you selected "Research design and plan" in question 13, please choose the most appropriate patients' activities in this step.	Implement the step n.4 with examples of patient engagement activities	7 steps R&I path
Q18	Additional	If you selected "Research Execution" in question 13, please choose the most appropriate patients' activities in this step.	Implement the step n.5 with examples of patient engagement activities	7 steps R&I path
Q19	Additional	If you selected "Research Evaluation" in question 13, please choose the most appropriate patients' activities in this step.	Implement the step n.6 with examples of patient engagement activities	7 steps R&I path
Q20	Additional	If you selected "Translation to the community" in question 13, please choose the most appropriate patients' activities in this step.	Implement the step n.7 with examples of patient engagement activities	7 steps R&I path
Q21	Core	<p>Methods to integrate patients' experiences and knowledge.</p> <p>What method can facilitate patients in providing their experiences of the disease and make them usable for research purposes?</p> <p>You can read information about the methods here (an explanation of method available for download). Please choose up to three.</p>	Gather approval and prioritization on the proposed methods for effective patient engagement (focus on integrating patients' experiences and knowledge in R&I), and identify additional methods.	Methods to integrate patients' knowledge in R&I
Q22	Core	<p>What do you think is the best way to measure the real effectiveness of patient engagement in the research process?</p> <p>(Please select up to 3 options)</p>	Gather approval and prioritization on the proposed metrics to assess the effectiveness of patient engagement, and identify additional metrics.	Effectiveness of patient engagement
Q23	Core	Is there anything else that you would like to contribute to this survey?	Gather any other feedback that may be helpful in designing the guidelines and toward next steps of the WP1 activities. Give the respondents the possibility to express their view on the survey.	Closing remarks
Q24	Core	If you wish, you can provide your e-mail address below to allow the consortium to contact you if they want to learn more about your experiences and suggestions? Moreover, if you wish to subscribe to the MULTI-ACT newsletter (2 per year) please visit our website.	Give the respondents the possibility to be contacted and to sign to the MULTI-ACT newsletter.	Closing remarks



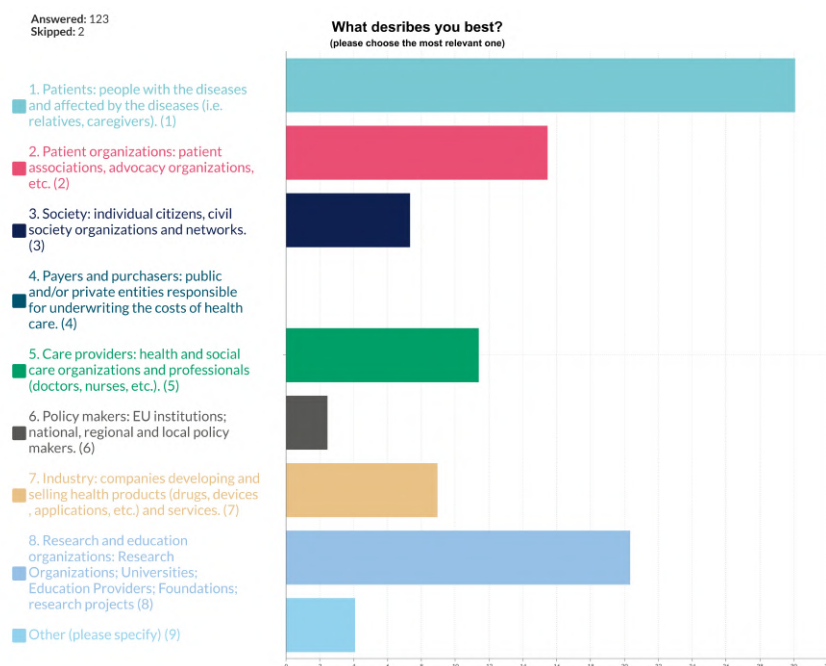
Results & Key findings

The total respondents who completed the survey were 125.

Demographic background of the study population (Q2-Q5)

The largest respondent (stakeholder) group was the category **Patients** [30.08% (n=37)], followed by **Research and education organizations** [20.33% (n=25)], **Patients organizations** [15.45% (n=19)], **Care providers** [11.38% (n=14)], **Industry** [8.94% (n=11)], and **Society** [7.32% (n=9)]. The groups not represented is the category **Payers and purchasers** [0% (n=0)]. The different representation between the responder categories has been taken into account while drawing conclusions especially for the groups with the lowest representations (*Figure 1: Stakeholder respondents*).

Figure 1: Stakeholder respondents



The age of respondents ranges mainly from 35 to 64 years old.

Respondents completed the survey from n.34 countries. The majority of responses originated from the countries of the consortium and the associated networks: **France** [16.38% (n=19 + 1)], **Belgium** (EBC) [15.32% (n=19 + 1)], **Italy** (FISM) [15.32% (n=19)], **UK** (MSIF) [12.10% (n=15)], **Spain** UBU [6.45% (n=8)], **Finland** UTA [4.03% (n=5)], **Greece** INTRA [2.42% (n=3+1)]. The group 'Other' comprises n.6 countries (United States, Canada, Russia, Australia, Norway, Bosnia and Herzegovina), both within and outside the European Union. There were an high number from the US and Canada, also because US and Canadian MS Societies are members of the MS International Federation Research Staff Network deeply involved in Patient Engagement.

The respondents were unbalanced in terms of gender: **Female 66.94%** (n=81), **Male 32.23%** (n=39), **X = 0,83 %** (n=1). This can be associated to the fact that the majority of respondents for the Patient category [30.08% (n=37)] is probably representing patients with Multiple Sclerosis (MS), the case-study disease of MULTI-ACT. MS is now universally found to be more prevalent in women than men, a phenomenon shared with several other autoimmune diseases. Furthermore, the MS prevalence ratio of women to men, that has increased markedly during the last decades, is (2.3–3.5:1).¹

¹ Jobin C, Larochelle C, Parpal H, Coyle PK, Duquette P: Gender issues in multiple sclerosis: an update. *Womens Health (Lond Engl)* 2010, 6(6):797-820

Experience of patient engagement (Q6-Q7)

The majority of respondents had previous experience of patients engagement [79.03% (n=98)].

Some of the reported experiences are presented below:

"My PhD is about eliciting patient preferences to inform decisions throughout the drug life cycle. In that context I am setting up two patient preference studies to make recommendations towards healthcare stakeholders on how to best elicit preferences. Research partners are patient organisations and patients from lung cancer and multiple myeloma patient groups. Their contributions are very useful; ensuring the protocol answers patient relevant research questions, ensuring questions in the preference survey are understandable to patients, ensuring patient needs in general (e.g. communicating results back to patient community) are met."

"This is the collective response of many doctors across the EU. As healthcare professionals, a number of our members are involved in the establishment of patients' organizations in various countries e.g. "Keratoconus" in Denmark. Some are board members of such associations and respond to questions raised on the web sites of the organisations. Engaged also in research in patient engagement with patients being involved in translation a PROM from English to the target language for the various countries. The board of such organisations also answers questions raised on the web-page etc."

"I was one of the founders of a patient forum where we aim to discuss the science around ME (www.s4me.info) in this forum we aim to get a mixture of patients (many of whom have a science background) and other scientists to discuss the latest research. I am also a member of a patient advisory group for a consortia of researchers looking to get more ME funding. In both I have seen genuine engagement between patients and the research community. As patients (or carers) we have also collaborated to publish papers."

"It has been mostly limited to RFCs by organizations and so far has been very underwhelming, the substance of our input is largely dismissed."

"8 years representing a support group, dealing with local medical authorities; the 'officials' were disingenuous and wasted the time and well-being of me and others. Nothing positive to report."

"As an Adviser at the Research Section I have been responsible for User Representatives who participate in the assessment of research projects being proposed for funding from the Norwegian Cancer Society since 2016. The main premise for me (us) is that user involvement positively impacts the quality of health care and contributes to research that is more relevant. The purpose of user involvement is to ensure the quality of cancer research and to make the research more accessible through user involvement. Research is one of the Norwegian Cancer Society's main priorities, and we are the largest financial contributor to cancer research outside the public sector. In 2018, we allocated NOK 235 million to cancer research. Fields of research is broad: From more basic, to translational and also more Close to the patient; clinical and Epidemiological, health, and social science cancer Research."

"Patient involvement in guideline development; It's not only useful, a necessity if you want your guideline to be used; also if you want to get your guideline validated by the national committee, it is necessary to involve users."

The main experiences of patient engagement reported are:

- facilitators of patient engagement in project design for better services in health care,
- recruitment of patients as end-users, engagement of patients in sharing of research results and in the research steering committees,

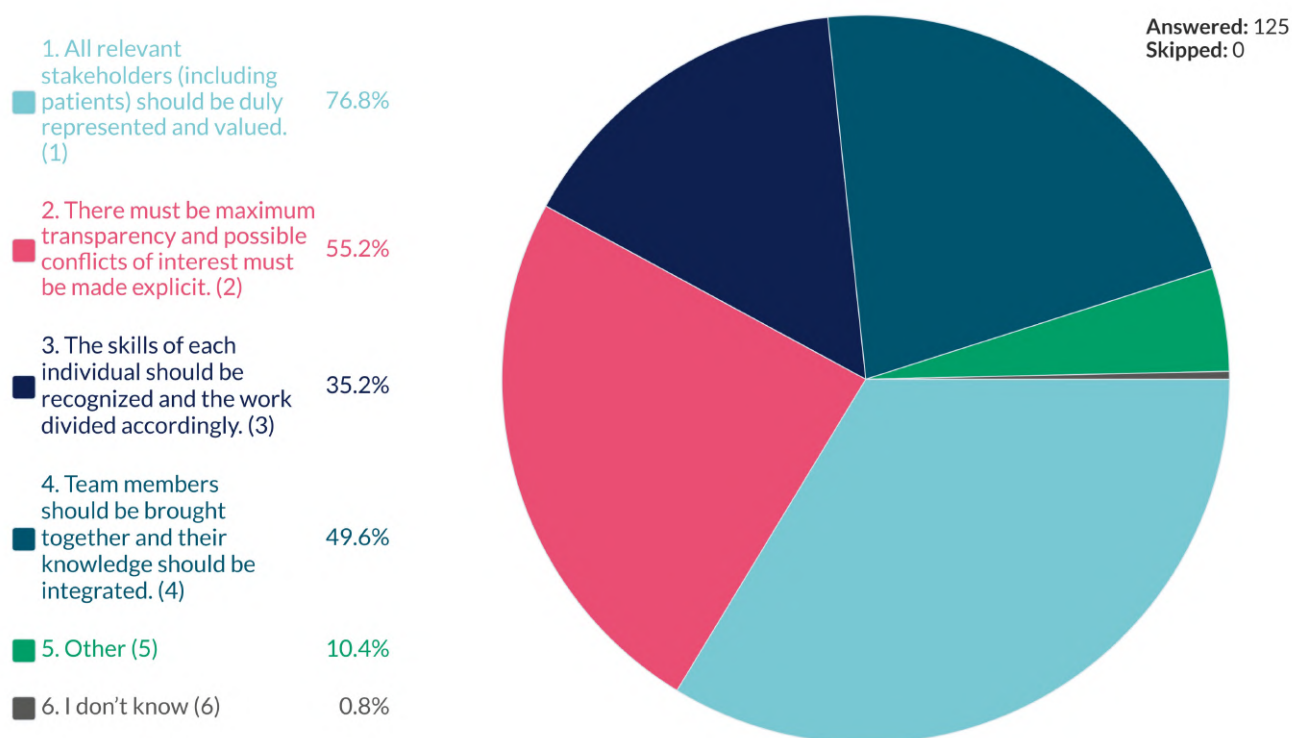
- engagement in the pre-design phase of a medicine development protocol as [EUPATI](#) fellow, coordination of a network of volunteers involved in reviews of research funding application,
- engaged in the co-development and co-creation of tools to support patient doctor communication,
- facilitation of patient focus groups to understand unmet support needs (patients and carers),
- development of patient speaker programme to raise awareness and educate pharma on disease impact,
- input into strategic planning, insight of patient experience in clinical department,
- engagement as patients in evaluating and developing services and in users committee,
- working for project dedicated to enabling patient engagement and capturing patient preferences,
- experiences of writing with patients a consent form, working in patient organizations as a person with a chronic disease, supporting clinical teams in engaging with patients in Research & Development (R&D) activities such as collaboration with patient advocates in designing protocols,
- co-creating accessible document templates and collecting their insights, coordination of a “patient on campus”,
- activities to inform patients, etc.

Assumptions for patient engagement (Q8)

This paragraph analyses the additional comments (n=17) to question n.8 ***“The main task of the Engagement Coordination Team is to make it possible for patients to be genuinely engaged, to ensure representativeness of patients’ contribution, to motivate them to participate, to bring out their experiences that are useful for research, to make the results of the research comprehensible to all and to stimulate feedback. Which of the following statements do you consider most appropriate for the purpose?”***

Although the majority prioritize the concept that “All relevant stakeholders (including patients) should be duly represented and valued” [76.80% (n=96)], all the statements have been found relevant (Figure 2: Engagement Coordination Team).

Figure 2: Engagement Coordination Team



Comments highlighted the fact that patient engagement should **not be a mere ticking the box**:

- I. Ticking the Box may be easy but when real patient engagement happens, pitfalls and challenges arise. Those should be anticipated and taken into account.
- II. *"The actual substance of patient input has to be taken into account, not merely a part of the process that ultimately has no impact."*
- III. It's important to have a feedback loop *"stakeholder input should be communicated back to show how knowledge has been integrated and the resulting actions developed"*
- IV. Patients should have influence on research outcomes and results.

Comments highlighted the importance of **recognition and rewarding**:

- V. The expertise of patients should be recognised and compensated as for other types of experts. Patients' participation should take place from the start and should be planned and earmarked in the research budget.
- VI. Patients should be considered equal contributors to patient-centred research - both in terms of the work that they do but also in terms of compensation. Most patients contribute just out of altruism and a desire to make systematic change, *"we cannot take advantage of their time and skills, they must be fairly compensated and we must see "patient representative" as a job in the team and not free labour"*.

The importance of **training**:

- VII. Co-learning protocols should be planned in order to identify shared needs.
- VIII. Engagement and involvement is about relationships, researchers need to understand that involved patients need support, training, payment and respect and to be treated as a 'full' member of teams.
- IX. Support and training should be provided to build the capacity of the patients to bring the best value from the engagement and involvement.

The importance of patients' **decision making role** and to duly recognize the **relevance** of patients' contribution:

"If stakeholders (e.g. patient representatives) truly should have influence on the course and results of research, it is not enough to consider their opinions, stakeholders really need to be allowed to make important decisions in the areas where their view is decisive. If they are not able to decide at once, they need training to do so. Otherwise the researchers/project management, which most often consists of research professionals, will always believe that they have the most convincing arguments and overrule the stakeholders."

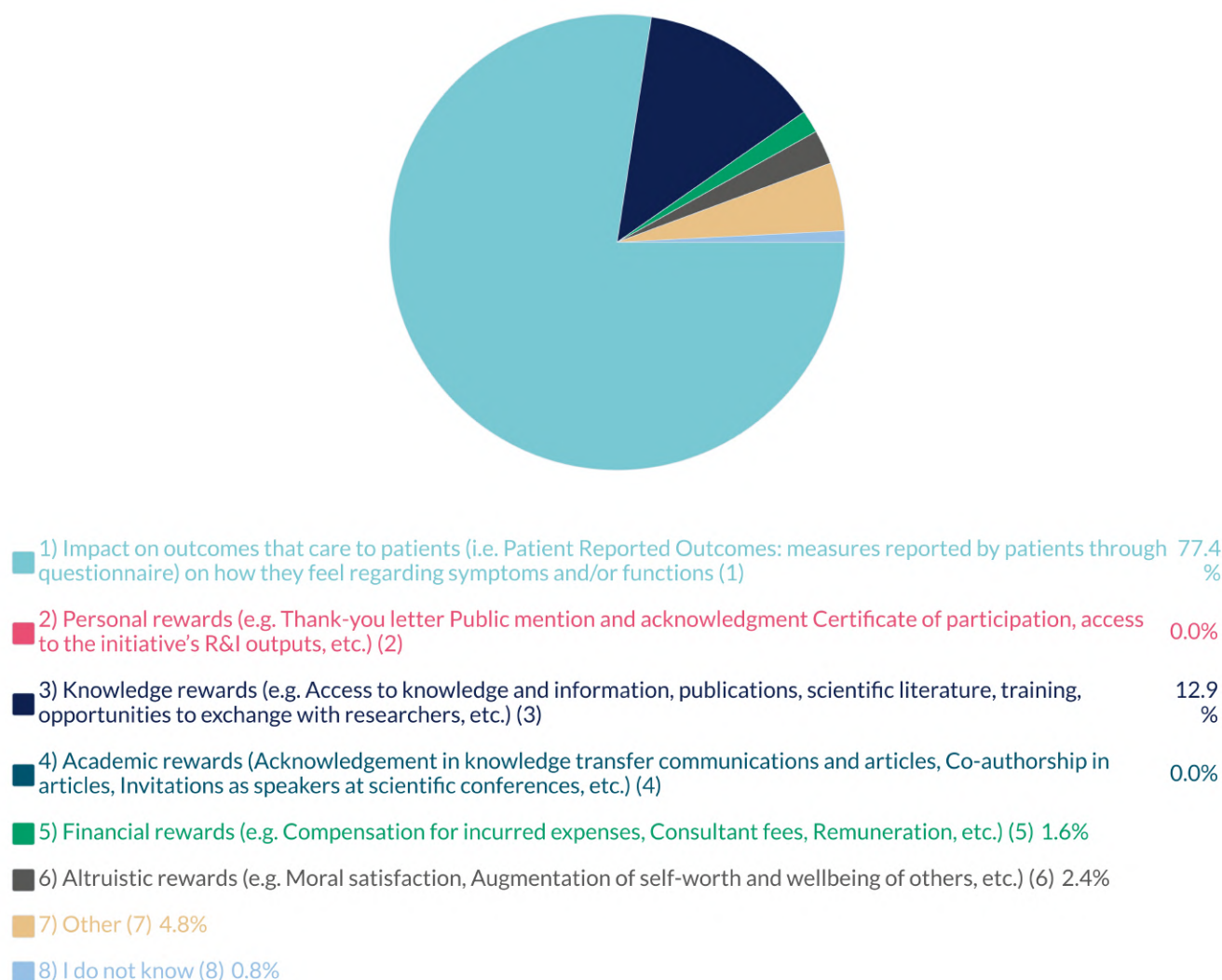
Other relevant aspects emerged from the comments:

- XI. Equitable access to medicines.
- XII. Increase contact with clinicians and value the experience of patients.
- XIII. *"It is important that patients help researchers understand what the disease is."*
- XIV. All parties should be vigilant and aware when their activities, statistics and studies cross over those of others and if appropriate ensure sharing of information is undertaken to support all.

Benefit of patient engagement (Q9)

This paragraph analyses the additional comments (n=11) to questions n.9 “**What is the most important benefit of engagement in scientific research and innovation for patients?**”

Figure 3: Benefit of patient engagement



The majority reported that the most important benefit of engagement for patients is having “Impact on outcomes that care to patients (i.e. Patient Reported Outcomes: measures reported by patients through questionnaire) on how they feel regarding symptoms and/or functions” [77.42% (n=96)] (Figure 3: Benefit of patient engagement).

Comments to this question highlighted the importance of **patients’ trust** “Is patient engagement a mere paper exercise?”.

The most important benefit must be that the engagement makes the results from research **better for the future patients and contributes to patient outcomes**.

Patients can develop **extensive knowledge** and collaboration can lead to an improvement in the research and its methodology, including **selection of criteria and outcomes**.

User/patients engagement clarifies the responsibility of researchers towards society. A dialogue between researchers and user/patients representatives gives:

1. Awareness to researchers in terms of explaining their research objectives to the patient/layman;
2. Increased understanding of the design, purpose, goal and future impact of the research,
3. Better communication of the research findings to the patient community/organizations.

As emerged also for the question n.8, comments highlighted the need to avoid tokenism, *“A genuine interest in the patient’s experience and building the research, diagnostics, treatment and care in order to improve the patients well-being”*.

One comment stated that all of the mentioned options may be relevant reasons for being involved while another one stated that it is more important to focus on human values and not on external rewards like personal, financial etc.

Some comments focused more on medicine development:

- *“More fully informed protocol development. Given that only 11.3% of all clinical trials are completed, mostly due to trial design, designing protocols with input from potential participants’ perspective is valuable. This co-creation helps ensure protocols that can actually be managed by participants, increase the possibility of trial completion, improves the opportunity for real world data and experience thus better preparing the drug or device for market.”*
- *“While we do research for “new treatments” we should get insight in the effectiveness of those we already use.”*

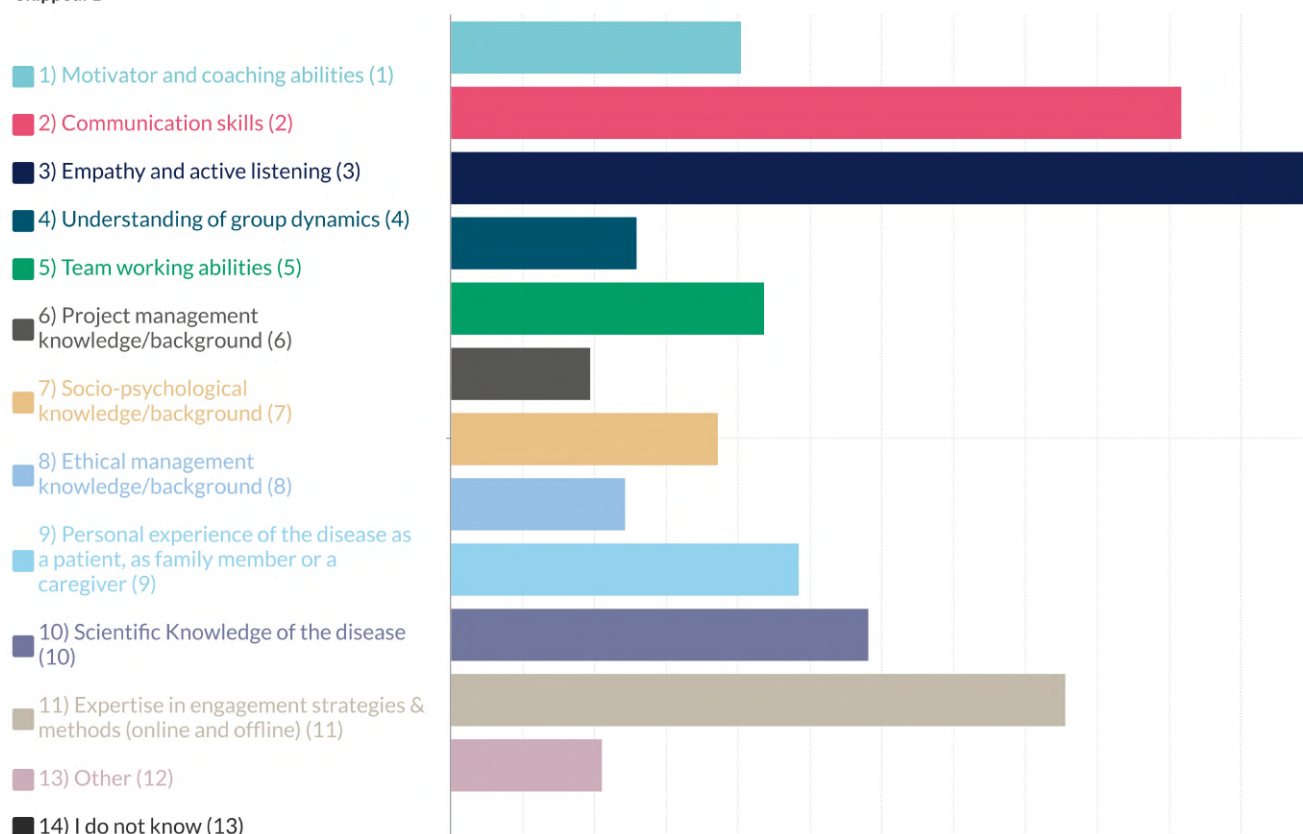
Skills of the Engagement Coordination Team (Q10)

This paragraph analyses the additional comments (n=12) to questions n.10 ***“The patient’s disease experience is of enormous value in directing research towards relevant goals. This experience is composed of many different aspects: needs, feelings, uncertainties, stories, reflections, insights, etc. The task of the Engagement Coordination Team is to translate these inputs into an experiential knowledge useful for research. What skills do the Engagement Coordination Team’s members need most to enable patients make their experience valuable for research?”***

The three main skills prioritized for the ECT are **“Empathy and active listening”** [59.68% (n=74)], **“Communication skills”** [50.81% (n=63)] and **“Expertise in engagement strategies & methods (online and offline)”** [42.74% (n=53)] (Figure 4: Skills of Engagement Coordination Team).

Figure 4: Skills of the Engagement Coordination Team

Answered: 124
Skipped: 1



Comments to this question highlighted some additional skills such as:

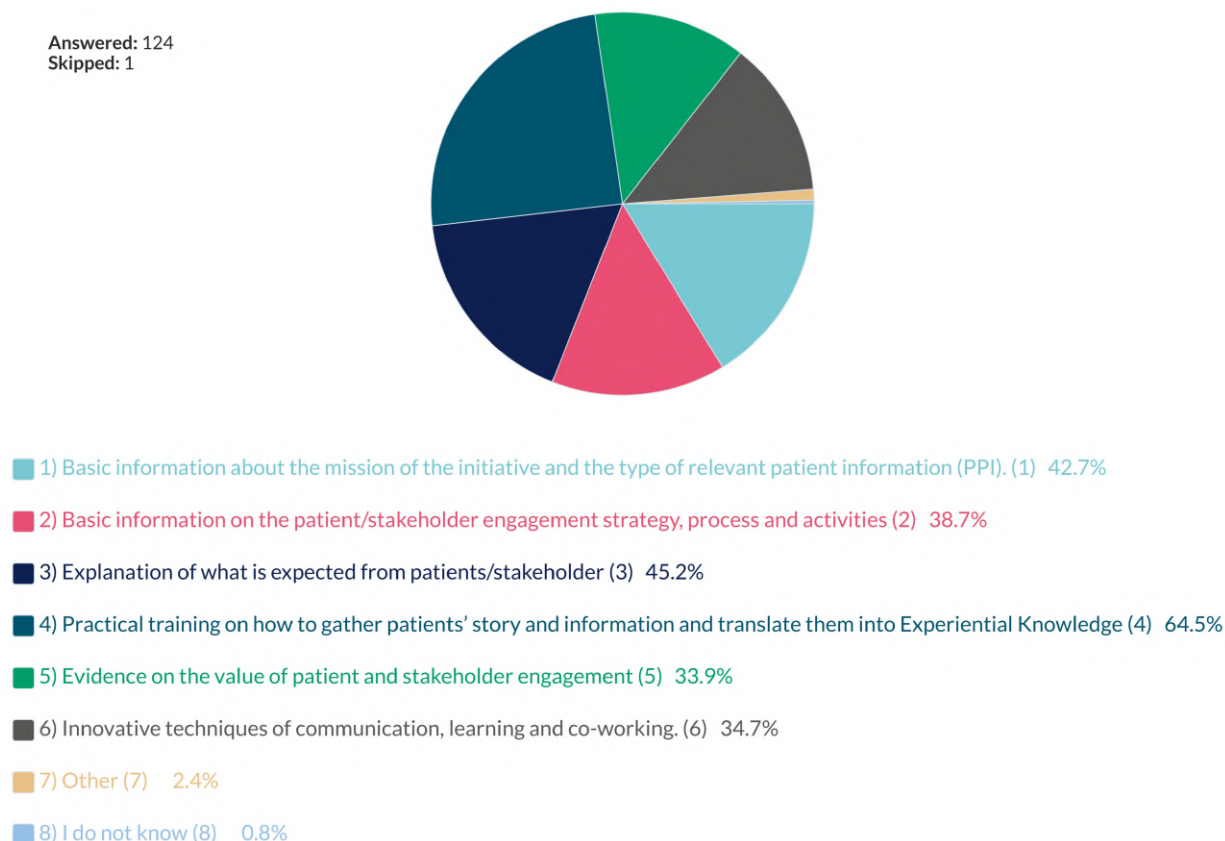
- Negotiation skills and innovative thinking.
- Absence of bias and prejudice, which will ultimately dismiss patient input.
- Empathy plus compassion; Knowledge, experience and willingness to take a comprehensive history.
- An ability to talk to different audiences in a way that is relevant to all stakeholders, and to 'translate' across boundaries (e.g. academics speak and understand very differently than patients; clinicians are different compared to academics etc.).
- Know how the patient community works, from Association, to forums, advocates and Facebook groups.
- Competences in social science research methods. Even patient's attitudes need to be examined scientifically, best in a multi-method approach including quantitative and qualitative research.
- Merge different abilities and assure a good general background knowledge.

Training for the Engagement Coordination Team (Q11)

This paragraph analyses the additional comments (n=7) to questions n.11 ***“What is the most suitable training for the Engagement Coordination Team to integrate patient experiential knowledge in research?”***

The three main features prioritized for the ECT are **“Practical training on how to gather patients’ story and information and translate them into Experiential Knowledge”** [64.52% (n=80)], **“Explanation of what is expected from patients/stakeholder”** [45.16% (n=56)] and **“Basic information about the mission of the initiative and the type of relevant patient information (PPI)”** [42.74% (n=53)] (Figure 5: Training for the Engagement Coordination Team).

Figure 5: Training for the Engagement Coordination Team



Comments to this question highlighted that training modules should use plain language and the content should be kept simple and respect for the human rights and the dignity of the patient should always be considered when designing a training program.

Information on methods for patients' engagement should be integrated with examples of application in real cases for each method.

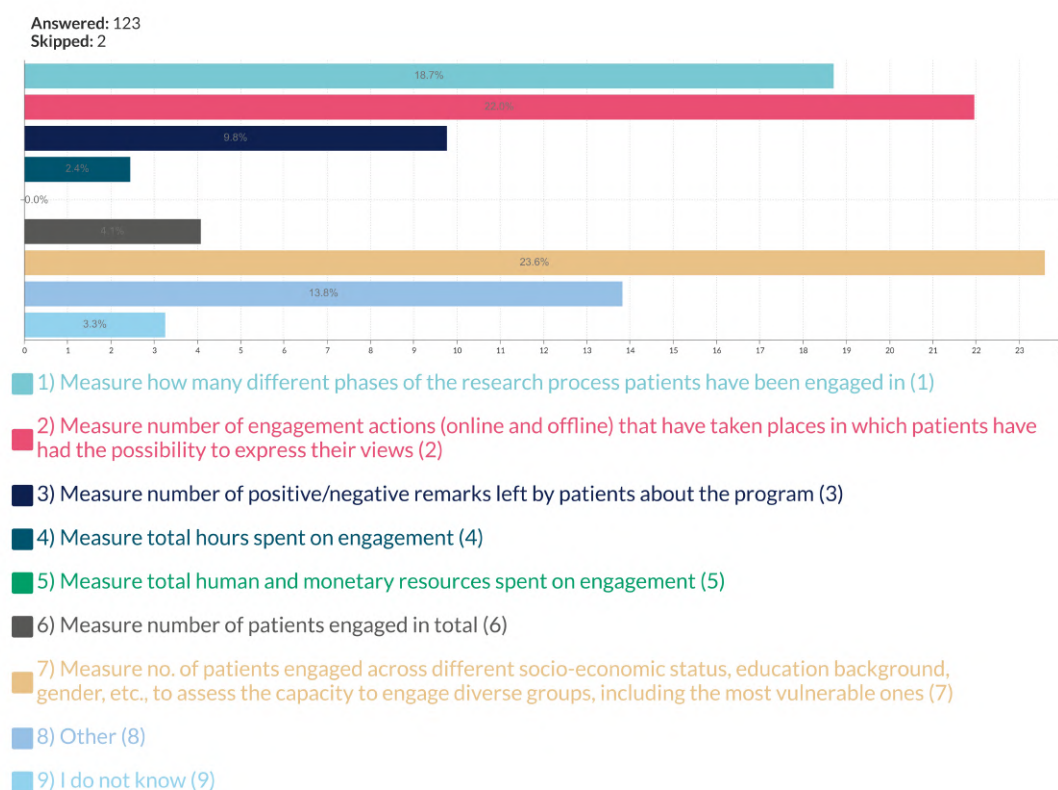
Moreover, it is important to include in the training modules an explanation of what are the expected outcomes of the multi-stakeholder initiative and how do those outcomes relate to the patients' needs in the given disease area.

Measuring the performance of patient engagement (Q12)

This paragraph analyses the additional comments (n=21) to question n.12 ***“What is the best way to measure the performance of patient engagement in research?”***.

The three main options prioritized for measuring the performance of patient engagement in research are **“measuring the number of patients engaged across different socio-economic status, education background, gender, etc., to assess the capacity to engage diverse groups, including the most vulnerable ones”** [23.58% (n=29)], **“measuring the number of engagement actions (online and offline) that have taken place in which patients have had the possibility to express their views”** [21.95% (n=27)], and **“measuring how many different phases of the research process patients have been engaged in”** [18.70% (n=23)] (Figure 6: Performance of patient engagement).

Figure 6: Performance of patient engagement



The survey protocol included two questions on assessment of patient engagement, one on the performance (Q12) and the other for the impact, effectiveness and value (Q21). Considering that the question on impact (Q21) arrived at the end most of the respondents anticipated the concept that not only performance should have been assessed but also the impact.

"To measure the performance of patients in research you need to co-create tools to do this measurement. It's more than a matter of just telling stories or looking at how many hours the patient spent you have to look at him meaningful the engagement was to the patients as well as to the research and the research team. I keep seeing this term more research needs to be done to determine the value of patient engagement."

In fact, many comments were directed more to the assessment of the **impact, effectiveness and value of engagement** rather than performance and they have been taken into account for the purpose of question Q21 (Methods to integrate patients' experiential knowledge in R&I (Q21)).

The highlights that we consider more relevant for the performance assessment are:

PERFORMANCE ASSESSMENT (PA)

PA - Quantitative:

- Measure the number of KPIs able to assess the impact of patient engagement.
- Measure the number of trainings conducted.
- Measure the extent into which the patient involvement at the end is implemented in the research path.
- Measure the number of interviews about patients' experience in the engagement process.
- Measure the number of co-created tools.

PA - Qualitative:

- Analyse if the patients' expectation with respect to the patient engagement are met.
- Analyse if patients felt engaged.
- Analyse how meaningful the engagement was to the patients as well as to the research and the research team.

PA - Methodological:

- Integrated top down approach from sources like technology wearable data create a quicker way to measure engagement.
- The analysis of how patients have been engaged (e.g. collecting comments, surveys, feedback, etc.).

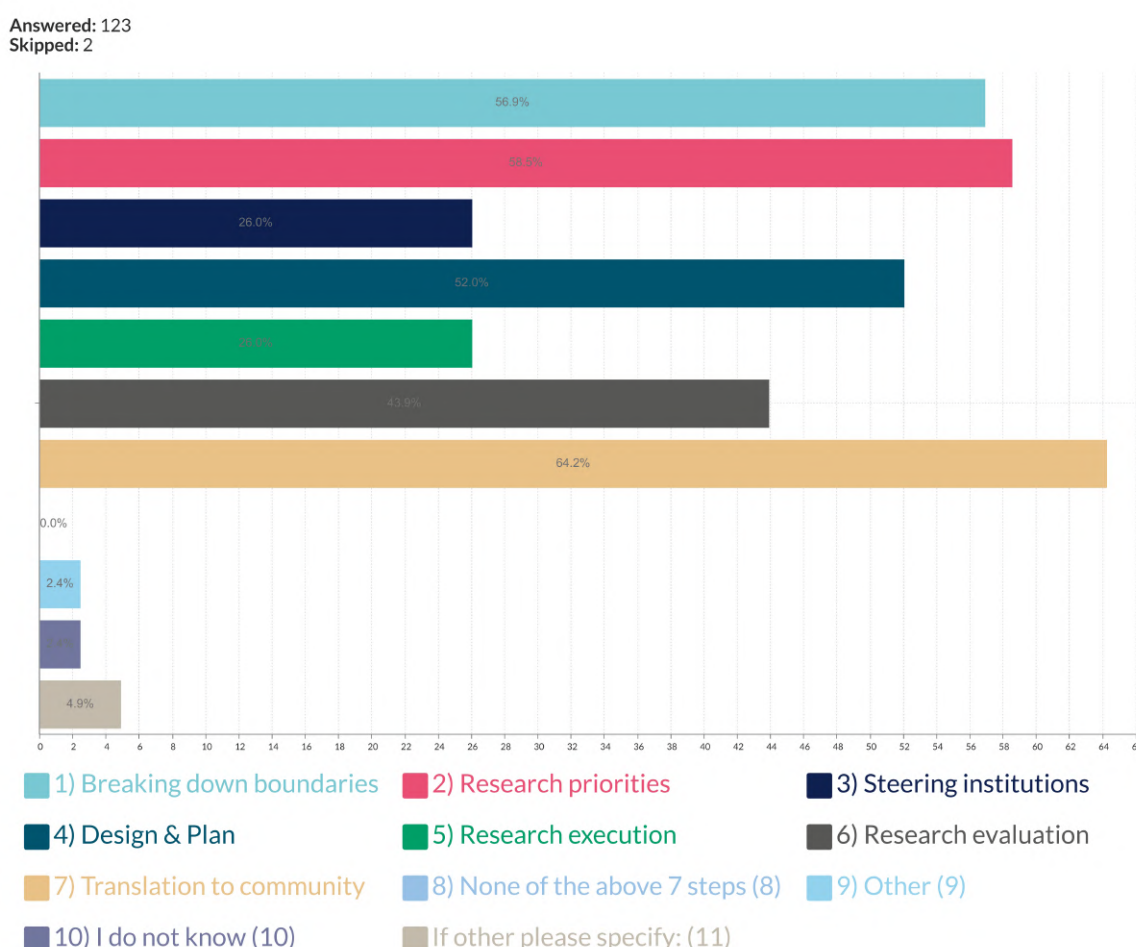
🔍 Patient engagement in the 7 steps R&I path (Q13)

This paragraph analyses the additional comments (n=6) to question n.13 ***“Features of patient engagement in research. In MULTI-ACT, patient engagement should cover every step of the research and innovation activities. We have identified 7 steps in which patients should be engaged with other stakeholders in this process, according to the mission and objectives of the program and/or project. At which step of the research process do you think patients should be engaged?”***.

The steps have been found more or less all relevant (>20% responses for each step), in line with the MULTI-ACT proposal that “patient engagement should cover every step of the research and innovation (R&I) activities.”

The steps prioritized (>50% responses) are **“Translation to community”** [64.23% (n=79)], **“Setting research priorities”** [58.54% (n=72)], **“Breaking down boundaries between patients and stakeholders”** [56.91% (n=70)], **“Research design and plan”** [52.03% (n=64)], **“Research evaluation”** [43.90% (n=54)], **“Steering institutions”** and **“Research execution”** [26.02% (n=32)] (Figure 7: Patient engagement in the 7 steps R&I path).

Figure 7: Patient engagement in the 7 steps R&I path



Legend - Figure 7: Patient engagement in the 7 steps R&I path

1) Breaking down boundaries between patients and stakeholders: conditions that should be set in order to facilitate patient engagement as standard practice in funding and research organizations (1)

2) Setting research priorities: all actions necessary to prioritize the Research Agenda and formulate its objectives. (2)

3) Steering institutions: all actions performed to establish steering and advisory committees and boards for providing guidance on company policy and objectives, budgetary control, marketing strategy, resource allocation, and decisions involving large expenditures, etc. (3)

4) Research design and plan: the design of all the activities that lead to the realization of a concept or idea (i.e. the formulation and assignment of tasks, rules, roles and execution times in order to achieve the objectives set, etc.). (4)

5) Research execution: all activities to implement a research program or a specific project in order to achieve the objectives set. (5)

6) Research evaluation: all activities to determine the value of a research program or project, establishing their outputs and results, the degree of achievement of its objectives and its impact. (6)

7) Translation to community: dissemination and communication activities to promote the adoption of the results of research programs or projects, including all aspects of engagement in communication of results and advocacy (7)

In particular, some respondents (n=2) commented that patient engagement is likely to have greater impact when performed at earlier stages, rather than only in the late-stage.

Comments to this question highlighted that the steps where to engage patients' depends on the mission and scope of the engagement. Patients themselves may inform where they prefer to be engaged and where they think they can contribute most.

The patients most impacted by the disease are seldom engaged and mechanism should be put in place to ensure representativeness: the less impacted may help the engagement of the most impacted to break down boundaries between research and the underrepresented patients.

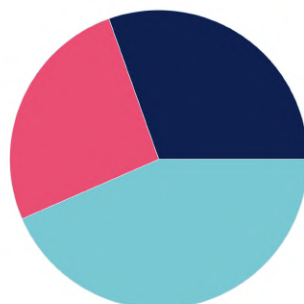
It is important to note that for the purpose of the Public Consultation, special attention is given to patients as key stakeholder. The scope of the MULTI-ACT Patient Engagement Guidelines is indeed to enable patients to be a stakeholder as the others in the group. In this transition phase where the paradigm is shifting toward enabling the "Science with/of Patient Input" and avoiding tokenism, the stakeholder "patient", final beneficiary of research outcomes, deserves special attention for an effective integration in the R&I processes. Those activities will lead to patients as equal stakeholders in R&I as a standard and usual practice, bringing knowledge and expertise complementary to the ones of the other stakeholders.

ANALYSIS OF EACH R&I STEP (Q14-Q20)

This paragraph analyses the comments to the questions from n.14 to n.20 ***"Please choose the most appropriate patients' activities in each step"***.

The proposed activities for each step have been found for the majority relevant (>20% of responses each proposed option).

Table 1: Patient Engagement activities along the 7-steps R&I path

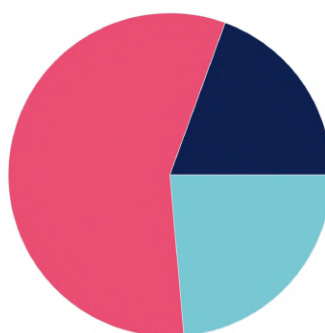
BREAKING DOWN
BOUNDARIES

- Patients help to define what are the boundary condition for patient engagement in multi-stakeholders (multi-SHs) initiative 40.0 %
- Patients help to review the policies and guidelines on how to engage patients in R&I activities 24.0 %
- Patients help to provide an overview on the facilities and infrastructures they need to be engaged in R&I 28.0 %

Additional activities suggested by respondents:

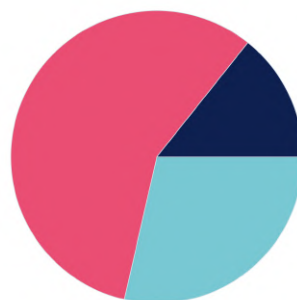
- Patients are engaged in recruitment and training of staff/research team
- Patients are engaged to identify commercially viable KPI that represent effectiveness from their perspectives.
- Patients are engaged to help the stakeholders to better understand the specificities of the patient community.

RESEARCH PRIORITIES



- Patients are engaged in advancing their interests in a specific research domain 23.0 %
- Patients are engaged to co-design research agenda 55.4 %
- Patients are engaged to exercise prioritization 18.9 %

STEERING INSTITUTIONS

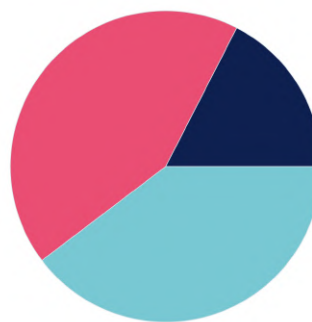


■ Patients are invited to advise steering and advisory committees 26.3 %

■ Patients are invited to be member of committees and boards to provide guidance on key issues such as company's policy and objectives, budgetary control, marketing strategy, resource allocation, and decisions involving large expenditures 52.6 %

■ Patients are engaged in defining ethical issues, anticipating risks and barriers for patient engagement in committee 13.2 %

DESIGN & PLAN



■ Patients are engaged to propose specific objectives of research 36.2 %

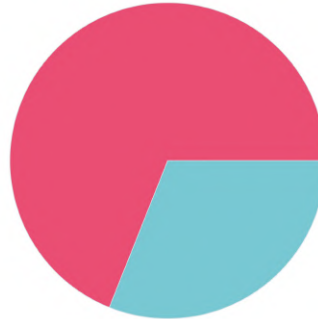
■ Patients are engaged to suggest endpoints and outcomes of research 39.1 %

■ Patients are engaged to discuss the design of work plan and budget of research 15.9 %

Additional activities suggested by respondents:

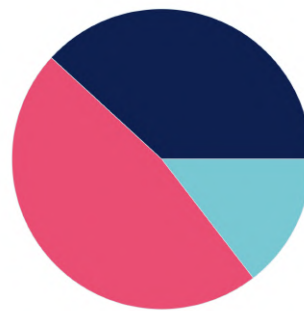
- Patients are engaged to define the relevance and acceptability of proposed research to patient community

RESEARCH EXECUTION

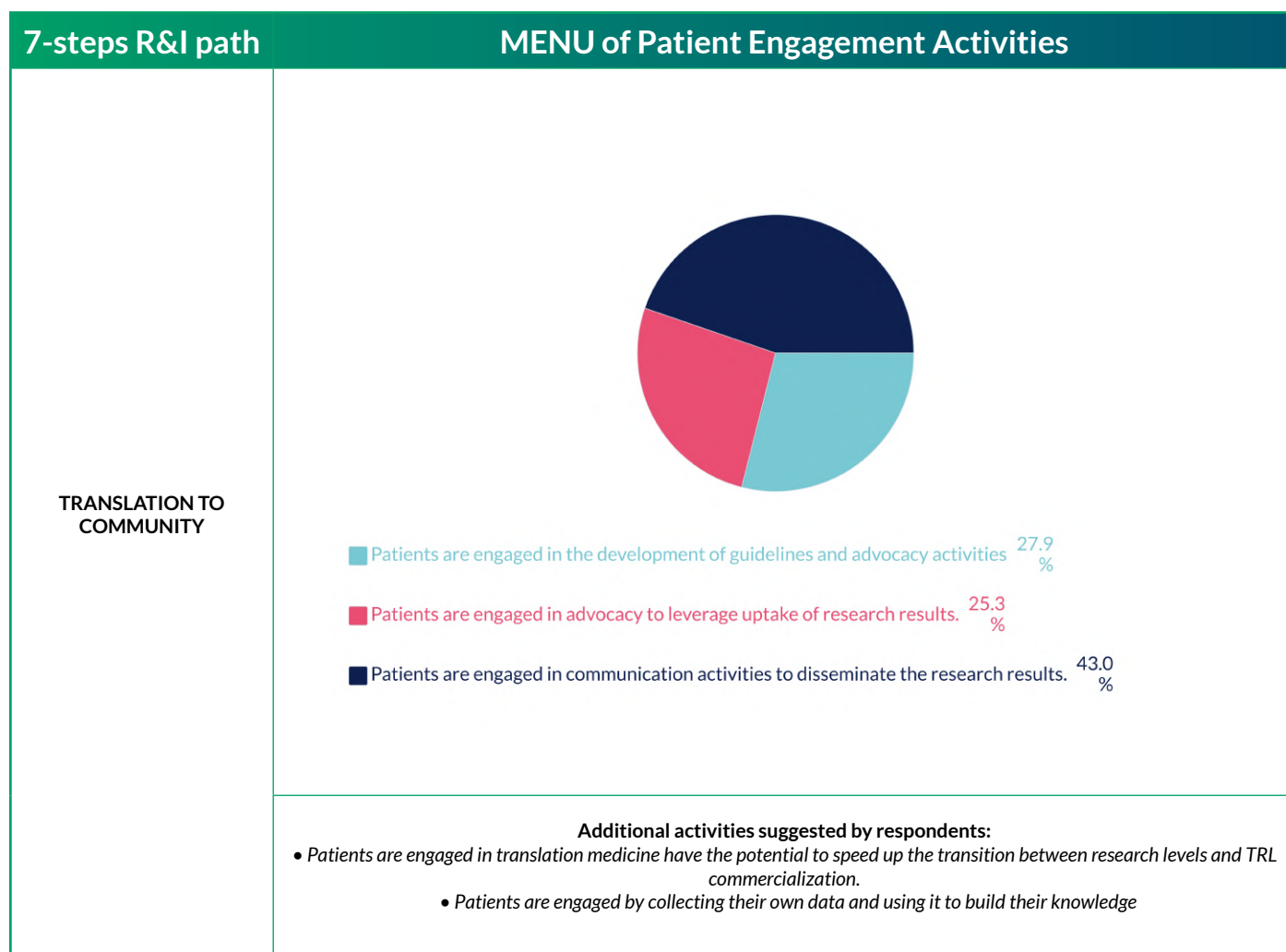


- Patients are engaged in the development and monitoring of research programs (e.g. release of call for proposals, selection of projects to be funded, monitoring of funded projects) 25.7 %
- Patients are engaged in the development and monitoring of research projects (e.g. collaborating for Information and Communication Technology 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) 57.1 %

EVALUATION



- Patients are engaged to working with other stakeholders on research reports 13.8 %
- Patients are engaged in discussing in the multi-stakeholder team about new methods to measure the impact of research 44.8 %
- Patients are engaged in the assessment of new approach and products arising from research 36.2 %

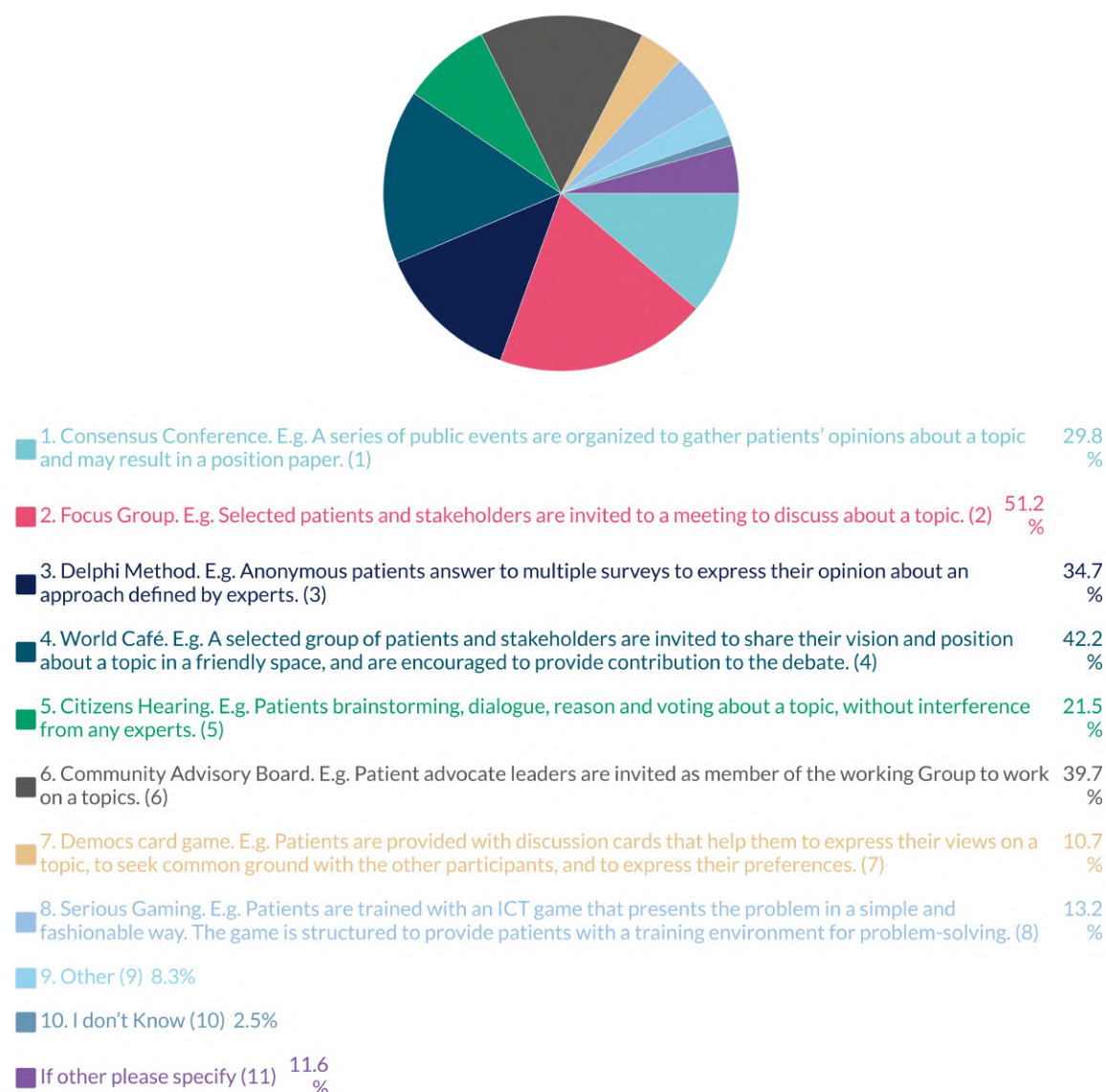


Methods to integrate patients' experiential knowledge in R&I (Q21)

This paragraph analyses the additional comments (n=14) to question n.21 ***“Methods to integrate patients’s experiences and knowledge. What method can facilitate patients in providing their experiences of the disease and make them usable for research purposes?”***.

The main preferences for methods to integrate patients’s experiences in R&I is the **“Focus Group. E.g. Selected patients and stakeholders are invited to a meeting to discuss about a topic.”** [51.24% (n=62)], followed by **“World Café World Café. E.g. A selected group of patients and stakeholders are invited to share their vision and position about atopic in a friendly space, and are encouraged to provide contribution to the debate.”** [42.15% (n=51)] and **“Community Advisory Board. E.g. Patient advocate leaders are invited as member of the working Group to work on a topics.”** [39.67% (n=48)] (Figure 8: *Methods to integrate patients’ experiential knowledge in R&I*).

Figure 8: Methods to integrate patients' experiential knowledge in R&I



Comments to this question suggested to use multi-channel approaches and to match and adapt the methods to the profile of the patients.

It emerged that Apps use may allow different levels of participation. Also “crowd sourcing areas of research direction using gamification and technologies” may be helpful.

Methods where patients talk such as online forums and support groups and discuss research can be effective.

The best method depends on the purpose of the engagement and – where possible - patients should be asked on their preferred methods for the purpose.

Also collecting data about symptoms and treatments is an engagement method. MULTI-ACT addresses this in the Science of patient Input.

The fact that experiences change by time, emotion, and circumstances should also be taken into consideration.

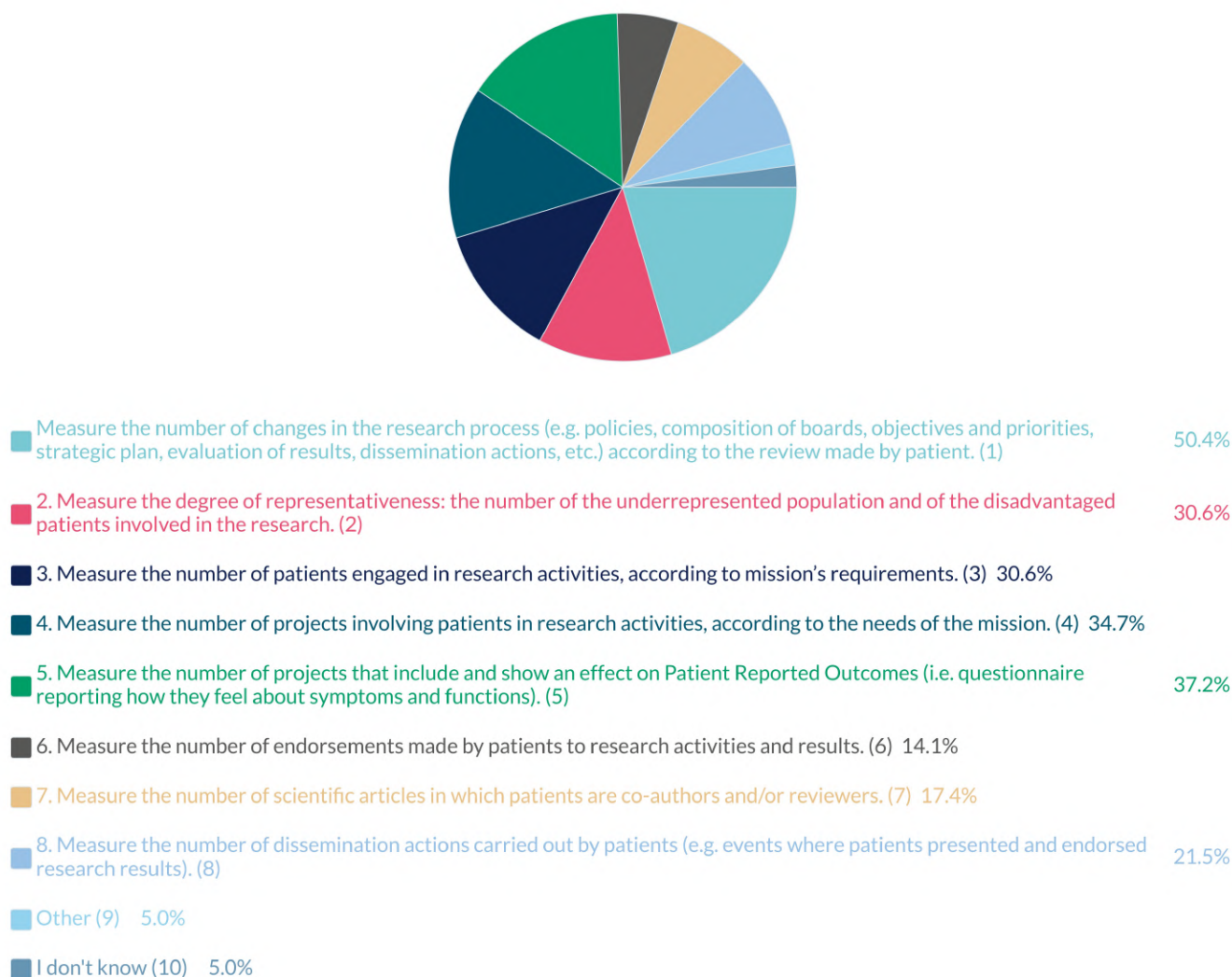
Scientifically sound surveys are representative for the target group, because they cover a large part of the stakeholders. Qualitative methods as those mentioned can complement this.

Measuring the effectiveness and impact of patient engagement (Q22)

This paragraph analyses the additional comments (n=7) to question n.22 ***“What do you think is the best way to measure the real effectiveness of patient engagement in the research process?”***

The three main options prioritized for measuring the effectiveness of patient engagement in research are **“Measure 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 patient.”** [50.41% (n=61)], **“Measure the number of projects that include and show an effect on Patient Reported Outcomes (i.e. questionnaire reporting how they feel about symptoms and functions).”** [37.19% (n=45)], and **“Measure the number of projects involving patients in research activities, according to the needs of the mission.”** [34.71% (n=42)] (Figure 9: Measuring the effectiveness and impact of patient engagement).

Figure 9: Measuring the effectiveness and impact of patient engagement



Comments to this question highlighted that the assessment should mix quantitative and qualitative evaluation, with slightly more focus on the qualitative one.

The assessment of effectiveness depends on the project's aims and time-frame.

As emerged from question n.8, it is important to assess the impact of patient engagement on “outcomes that matter to patients”.

In particular, the emerged measures, in addition to the n.8 options proposed by the survey, are:

IMPACT & EFFECTIVENESS ASSESSEMENT (IA)

IA - Quantitative:

- Measure the number of endorsements made by patient organisations.
- Measure the number of reality tests made by the patients.
- Measure the number of patient ‘interventions’ directly, or indirectly.

IA - Qualitative:

- Analyse if the lives of the engaged patients have been improved by the research.
- Analyse how patients’ lives may be or have been improved by the research.
- Analyse the long-term improvement in health indicators when engaging patients in the research process.
- Analyse if the value of patient contribution is the same as others stakeholders.
- Analyse if patients’ expectation with respect to the research and mission of the initiative are met.
- Analyse the achievement in terms of new knowledge produced by engaging patients in the research process, from the perspective of all the stakeholders.
- Analyse how patients have been engaged (e.g. collecting comments, surveys, feedback, etc.).
- Evaluation of the project plan, of all single project phases and of the final results by (trained) patient and if and how their suggestions have been integrated into the project activities.

Feedback for co-designing of the MULTI-ACT Patient Engagement Strategy (Q23)

This paragraph analyses the additional comments (n=22) to question n.23 ***“Is there anything else that you would like to contribute to this survey?”***.

The majority of comments are directed to the content of the survey, a minor part refers to methodological and technical aspects: n. 3 comments are constructive criticism on feasibility and n.1 comment is a methodological consideration.

The qualitative comments have been used to implement the strategy and the guidelines.

- to take into consideration country specific variations in performing engagement activities (methodological comment),
- to learn lessons from the cases where patient input has been rejected, to learn from mistakes and failures,
- to leverage on the testimony and experience of patients in addition to actual subject-matter experts,
- to manage prejudice and hostile behavior by the medical professionals towards the highly disabled patient population,
- to develop training to close the gap between patients and researchers, both ‘sides’ need to understand the issues faced by the other and how best to negotiate them (e.g. for staff, to understand the need for patient representation in research and how to go about it, for patients to help them understand what research is and how best to interact to represent patients). “We also need to stop talking about ‘sides’!”

- to listen to the patients and focus on quality,
- to keep the process simple,
- to engage patients in reviewing research proposals and in being part of the process of granting research proposals,
- to find the best way to engage patients as the feeling for researchers may be “as a PhD student I realise that I might want the patient to be too much in the loop”,
- to empower patients to deal with the disease instead of unconsciously becoming another one of their “harmers”.

Comments included a suggestion of sources for benchmarking growth on patient engagement (European Patients’ Forum developed a [framework](#) of evaluating the quality of patient involvement).

Some respondents reported (n=64) an interest in collaborating and provided additional feedback and left their contact details to know more about the project and to provide more experiences and suggestions, also including comments reporting enthusiasm and interest in the initiative, thanking us, wishing us good luck and looking forward to further steps (n=4).