multi-act

Deliverable D1.7

White Paper for innovative routes for patient engagement

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This document is a High-level policy-oriented document calling for actions to be taken by policy makers and R&I funders to foster patient engagement in Brain Health Research & Innovation (R&I).

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EXECUTIVE SUMMARY

According to a recent consensus document of the H2020 Scientific Panel for Health entitled 'Building the future of health research - Proposal for a European Council for Health Research', **"health, health research and health care has to form a unique and interdependent ecosystem. Healthcare cannot be separated from research. Health and health care are pillars of the social structure, and a public and societal responsibility"**[1].

To close the gap between science and society, the European Commission recommends and actively promotes the policy of Responsible Research and Innovation[2] (RRI).

A key feature of RRI, in addition to sound research ethics, is the involvement of different societal stakeholders throughout the research process. In health research particularly, RRI means to put the expectations of patients and society at the heart of research programs. The thinking that underlines patient engagement in RRI argues that excellence, validity and relevance are connected by engaging patients and society in the research continuum as key stakeholders[3].

On the other hand, it is now widely recognized that 'patient experiential knowledge', which is the lived experience of patients, complements the expertise of researchers.

However, the monitoring of the evolution and benefits of RRI has highlighted unmet needs in the area of patient engagement, among others.

The findings of MULTI-ACT clearly demonstrate that 'patients', as a key stakeholder category, are currently poorly represented in health R&I. Patients are very rarely involved in the governance of R&I according to the principles of RRI, and a clear/effective methodology for their engagement in R&I is lacking.

The MULTI-ACT analysis concluded that there is a need for clear and evidence-based methods for guidance on when and how to engage patients at all stages of R&I as well as a need for clear methodologies and metrics to assess the impact and (cost-) effectiveness of patient engagement in R&I. It is necessary to develop minimum quality criteria for the development, content, and governance of patient engagement.

In response to the identified needs, the MULTI-ACT patient engagement guidelines[4] were developed to empower health research teams to integrate patients' experiential knowledge in research and make patients key stakeholders with decision making roles, bringing to R&I expertise and knowledge complementary to the ones of the other stakeholders.

The MULTI-ACT Patient Engagement guidelines are relevant for all health research initiatives conducted by Health Research Funding and Performing Organisations (RFPOs), already formed as a

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[1] A consensus document of the H2020 Scientific Panel for Health / 15.05.2018. The Scientific Panel for Health (SPH) is a science-led expert group based on the provisions of the Horizon 2020 Specific Programme that has been tasked with helping to achieve better health and wellbeing for all. https://ec.europa.eu/programmes/horizon2020/en/h2020-section/scientific-panel-health-sph

[2] https://ec.europa.eu/programmes/horizon2020/en/h2020-section/responsible-research-innovation

[3] https://www.rri-tools.eu

[4] MULTI-ACT Patient Engagement Guidelines, short version v0.1 May 30th 2020: https://www.multiact.eu/project-deliverables/.

multi-stakeholder group (e.g. Alliance) or that are willing to start conducting their R&I with a multi-stakeholder and co-accountable approach to reach a transformational mission.

Policy makers and research funders, as decision makers in the support of Health R&I initiatives play an important role to foster the uptake of an RRI approach by RFPOs.

The MULTI-ACT Patient Engagement guidelines can be translated into tangible policy recommendations or requirements for RFPOs seeking financial support for their research. This would foster their uptake of an RRI approach which, in the case of health research, entails the inclusion of the patient perspective throughout the research continuum.

Policy makers[5] can do this in different ways:

1) to require RFPOs to conduct their R&I with a multi-stakeholder and co-accountable approach taking advantage of the MULTI-ACT Framework (i.e. tools and guidelines).
2) to require RFPOs of multi-stakeholder research initiatives, seeking to engage with patients, to use or adapt their patient engagement strategies to essential aspects of the MULTI-ACT Patient Engagement guidelines (these are detailed in the next section).
3) to ask Health R&I project applicants to provide a patient engagement strategy and an estimate of the cost to implement their patient engagement plan in their overall project application budget.
4) to provide adequate funding to support patient engagement activities in funded projects, including the application of the essential steps of the proposed patient engagement strategy.
5) to recommend the use of metrics to evaluate patient engagement actions in health research initiatives. It is suggested to use a mix of quantitative and qualitative metrics for the performance assessment of the patient engagement plan as well as for the effectiveness and value of patient engagement in R&I processes. MULTI-ACT has developed metrics to guide multi stakeholder brain research initiatives applying the MULTI-ACT framework[6].

These requests should be included in all applications for Health Research funding with dedicated questions in the application forms.

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[5] Policy makers have an important role in sharing and enabling the RRI approach among researchers and stakeholders.

[6] See Section "Measure the performance and effectiveness of patient engagement" of MULTI-ACT Patient Engagement Guidelines (page 11), version v0.1 May 30th 2020, and Deliverable D1.8 https://www.multiact.eu/project-deliverables/

1. INTRODUCTION

This document is the deliverable corresponding to the fourth task of WP1: “enabling the science with and of patient inputs” subsequent to the three tasks which culminated in the final version of the MULTI-ACT Patient Engagement in Health Research and Innovation (R&I) guidelines.

1.1 Purpose of this document

This document aims to put the MULTI-ACT Patient Engagement guidelines in the political context and to describe their alignment with policy objectives in the field of health research and innovation.

Based on the findings of the MULTI-ACT project on existing patient engagement experiences, it outlines a **call to action** for more effective patient engagement in health research and innovation.

The **call to action is addressed to policy makers and research funders** who, as decision makers in the support of Health R&I initiatives, can play an important role to foster the uptake of the MULTI-ACT Patient Engagement guidelines[7].

1.2 Structure of the document

This document is made of three sections:

1) Problem statement and contextual background

   This section outlines the context in which the guidelines were developed regarding patient engagement in research. It also outlines briefly the process for the guideline development with particular emphasis on the findings from the landscape analysis (gaps, needs, best practices and lessons learned from existing patient engagement experiences in brain health research) as a key milestone in this process.

2) MULTI-ACT Patient Engagement Guidelines. The guidelines are introduced with references for additional information.

3) Conclusions and Call to Action for effective patient engagement in responsible health research and innovation. This section summarizes the white paper’s major points and proposes ways to use the guidelines in supporting and financing health research and innovation.

1.3 Glossary

Please refer to D9.1 for classification and 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.

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[7] Please note that this White Paper is addressed to decision makers for R&I’s policies and funding, including public bodies and/or private research funding organizations, that aim to promote and fund a research co-created. and co-accountable toward patients and society. Example: European Commission, its Directorates and Agencies, National Ministries of Health, National Ministries of Research and Education, Regional and Local Health Authorities, Regional and Local Innovation Authorities, Authorities for Health Research funding, Clusters and Foundations funding brain diseases’ R&I, etc

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.

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." (Caron-Flinterman et al. 2005) 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"[8]. While various contexts use different terminology to describe the engagement process, such as patient and public involvement (PPI) in the UK[9], 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 patients[10].

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[8] 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.

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

[10] https://www.nationalhealthcouncil.org/wp-content/uploads/2019/12/NHC_Patient_Engagement_Rubric.pdf

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"[11]. 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.

Patient Reported Outcomes (PROs): "any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else" (FDA, 2009), "any outcome evaluated directly by the patient him/herself and based on patient's perception of a disease and its treatment(s)" (European Medicines Agency, 2014).

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).

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 (including RFPOs) process concerned with the governance and management of research funding & performing programs:
    - **Breaking down the boundaries** conditions that should be set in RFPOs 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.

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[11] https://www.eu-patient.eu/About-EPF/what-is-a-patient-organisation/

- **Project Level:** Project Development stages in Multi-stakeholder initiative (including RFPOs) 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. The Science *with* patient input will then 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[12] outside the clinic (Science of patient input) are critical to **evaluate** the impact of mission-oriented health research on outcomes that matter most to patients[13]. 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.

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[12] https://milkeninstitute.org/reports/advancing-models-patient-engagement-patient-organizations-research-and-data-partners

[13] 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.

Social media: forms of electronic/web communication (such as websites for social networking and microblogging) through which users create online communities to share information, ideas, personal messages, and other contents (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.

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". (Freeman, 1984)

- **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.

1.4 Abbreviations

Acronyms	
CRIF	Collective Research Impact Framework
PE	Patient Engagement
ECT	Engagement Coordination Team
PROs	Patient Reported Outcomes
PROMs	Patient Reported Outcomes Measures
R&I	Research and Innovation
RFPO	Research Funding and Performing Organization
RRI	Responsible Research & Innovation
ROI	Return on Investment

2. PROBLEM STATEMENT

2.1 Contextual background

According to a recent consensus document of the H2020 Scientific Panel for Health entitled 'Building the future of health research - Proposal for a European Council for Health Research', "health, health research and health care has to form a unique and interdependent ecosystem. Healthcare cannot be separated from research", Health and health care are pillars of the social structure, and a public and societal responsibility"[14].

To close the gap between science and society, the European Commission recommends and actively promotes the policy of Responsible Research and Innovation (RRI). RRI is defined as "an approach that anticipates and assesses potential implications and societal expectations with regard to Research and Innovation (R&I), with the aim to foster the design of inclusive and sustainable research and innovation"[15]. RRI has been highlighted throughout the objectives of the EU Horizon 2020 Programme.

In addition to sound research ethics, a key feature of RRI is the involvement of different societal stakeholders throughout the research process. It implies that societal actors (researchers, citizens, policymakers, business, third sector organisations, etc.) work together during the whole research and innovation process in order to better align both the process and its outcomes with the values, needs and expectations of society.

In health research particularly, RRI means to put the expectations of patients and society at the heart of research programs. The thinking that underlines patient engagement in RRI argues that excellence, validity, and relevance are connected by engaging patients and society in the research continuum as key stakeholders[16].

It is now widely recognized that 'patient experiential knowledge', which is the lived experience of patients, complements the expertise of researchers. However, the monitoring of the evolution and benefits of RRI has highlighted unmet needs in the area of patient engagement, among others.

2.2 Current issues for effective patient engagement

The development of the MULTI-ACT Patient Engagement guidelines followed an evidence-based process. They were driven by a mission-oriented research approach[17] where patients were engaged in research processes and decision making as a key stakeholder category to fulfil the mission.

A key milestone in this process was a landscape analysis of existing patient engagement experiences in brain research[18].

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[14] A consensus document of the H2020 Scientific Panel for Health / 15.05.2018. The Scientific Panel for Health (SPH) is a science-led expert group based on the provisions of the Horizon 2020 Specific Programme that has been tasked with helping to achieve better health and wellbeing for all. https://ec.europa.eu/programmes/horizon2020/en/h2020-section/scientific-panel-health-sph

[15] https://ec.europa.eu/programmes/horizon2020/en/h2020-section/responsible-research-innovation

[16] https://www.rri-tools.eu

[17] Mazzucato M (2018), Mission-Oriented Research & Innovation in the European Union A problem-solving approach to fuel innovation-led growth, EC Directorate-General for Research and Innovation , available at https://ec.europa.eu/info/sites/info/files/mazzucato_report_2018.pdf.

[18] 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/).

The landscape analysis, focused on brain disorders, was performed with different methodological steps: literature review, web research, interviews, surveys and connection with other relevant RRI initiatives. The collection of existing patient engagement procedures, experiences and best practices in R&I and a public consultation were instrumental to develop the MULTI-ACT Patient Engagement guidelines.

The web-search on mapping portals and European repositories found that experiences of patient engagement mainly concern adherence to medical treatments or participation in clinical trials and drug development, in which patients are often not engaged from the very beginning of the research with decision making role but consulted for feedback and drug testing.

The literature review found that the majority of studies focus on discussing experiences of engaging patients in healthcare and/or clinical research projects rather than engaging patients in the governance of wider R&I programs. So, patients are very rarely engaged in the governance of R&I according to the dictates of RRI.

The research projects in the health sector appear to have a rather limited repertoire of methods used for engaging patients. The methods are not discussed in terms of their (potential) impact. In conclusion, it was not possible to determine return on engagement[19] of the methods based on the literature.

The landscape analysis demonstrated that patients, as a key stakeholder category, are poorly represented in R&I and there is a need for clear and evidence-based methods for guidance on when and how to engage patients and the public at large at all stages of R&I.

The analysis concluded that it was necessary to develop minimum quality criteria for the development, content, and governance of patient engagement.

There is also a need for clear methodologies and metrics to assess the impact and (cost-) effectiveness of patient engagement in R&I as well as clear and constructive communication and professional management of stakeholders.

Take home messages from the landscape analysis were:

- The patients' experiential knowledge should not be confused with scientific/clinical knowledge, as it provides relevant and different insights on R&I[20];
- Patients, placed at the same level and with similar decision-making roles as researchers and other stakeholders, have the potential to maximise the impact of R&I and to assess it according to the results that matter most to them;

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[19] Return on Engagement: the benefit and impact resulting to 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 of metrics to measure the "return on engagement". The MULTI-ACT guidelines include a list of metrics to measure the performance and effectiveness of patient engagement in R&I.

[20] The engagement of people with and affected by the disease as partners with the "experience" of the disease across the continuum of research, development, and care is needed. Therefore, MULTI-ACT is attempting to strengthen the engagement of patients and public that may not have technical knowledge in R&D or regulatory processes ("expert patients" https://www.frontiersin.org/articles/10.3389/fmed.2018.00270/full), but that have the main role to contribute with their experiential knowledge of the disease across the continuum of health research and care.

- Patients organizations play a pivotal role in enabling the transition from individual to collective patients' experiential knowledge;
- Professional management of all stakeholders is effective in empowering patients' experiential knowledge in R&I, to better prioritize the needs of patients and society.

3. MULTI-ACT PATIENT ENGAGEMENT GUIDELINES

MULTI-ACT stems from the acknowledgement that stakeholder engagement in health research and innovation is an important pathway to achieving impact. It has created a new governance model allowing for the effective cooperation of all relevant stakeholders.

MULTI-ACT aims to fill the gaps identified in the landscape analysis by proposing a roadmap to capture ‘experiential knowledge’ of patients (see Figure 1), a knowledge that complements the expertise of researchers and that should be acknowledged and used as a valuable asset for research. The life experience of patients provides relevant and different insights on R&I which can potentially increase the impact of R&I on the outcomes that matter most to patients. This unique personal knowledge and experience can be applied to many stages of R&I, from planning research to reporting its results.

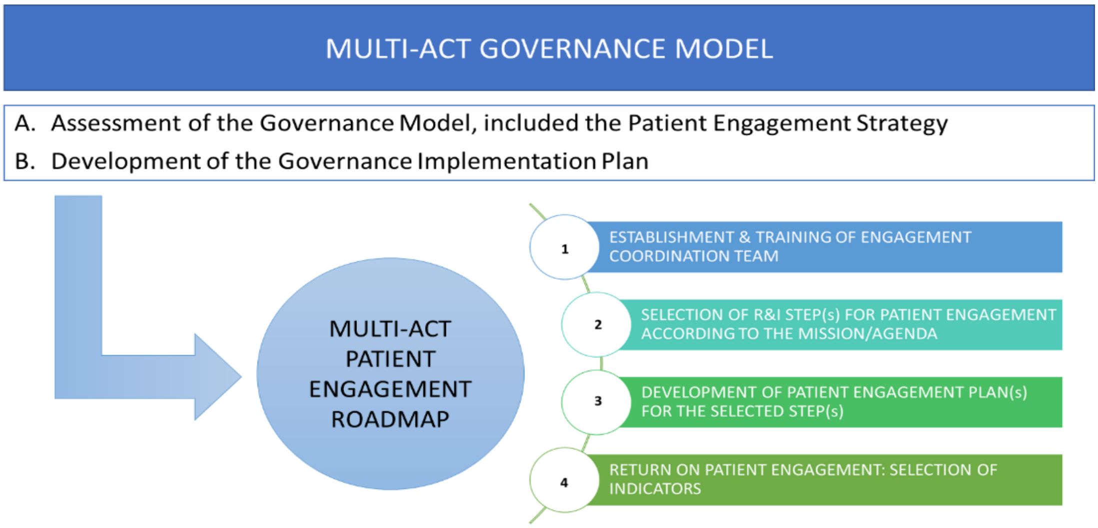

Figure 1. MULTI-ACT Patient Engagement Roadmap

While collecting patient data has always been important in brain diseases research, more fully engaging patients and caregivers in all relevant decision-making stages of a project can enrich the research, enhance its relevance, and ensure that a research initiative reflects the goals, or outcomes, that matter most to people affected by a disease[21].

In MULTI-ACT, the term “patients” refers to the persons with lived experience of the disease, and persons or groups that are affected by the disease, including family members and caregivers. The MULTI-ACT Patient Engagement guidelines[22] provide a strategy to empower the stakeholder “patients” to be engaged in R&I and to empower all the stakeholders to collaborate and co-create with the “patients”. The MULTI-ACT Patient Engagement guidelines are one of the three key components of the

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[21] The MULTI-ACT Patient Engagement Guidelines has been produced also in a “plain-version” targeting the language’s needs of patients and society that do not have a scientific background. The “plain-version” has been developed by the People affected by Multiple Sclerosis Engagement Coordination Team of the Progressive MS Alliance as result of the collaboration with MULTI-ACT.

[22] MULTI-ACT Patient Engagement Guidelines, short version v0.1 May 30th 2020, https://www.multiact.eu/project-deliverables/

MULTI-ACT framework alongside the Governance model[23] (Figure 2) and the co-accountability impact assessment model[24] (Figure 3). Measuring impact of health research on Patient Reported Dimension will maintain patients engaged as key stakeholder.

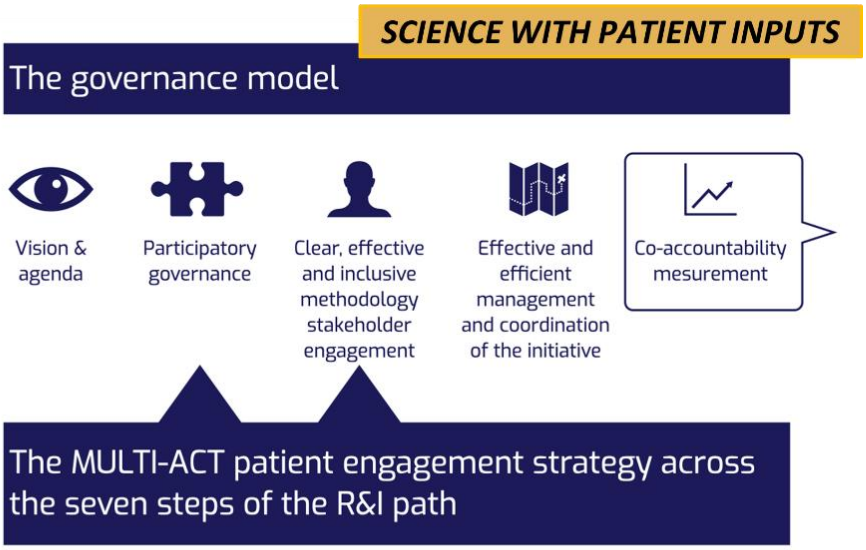

Figure 2. MULTI-ACT governance model

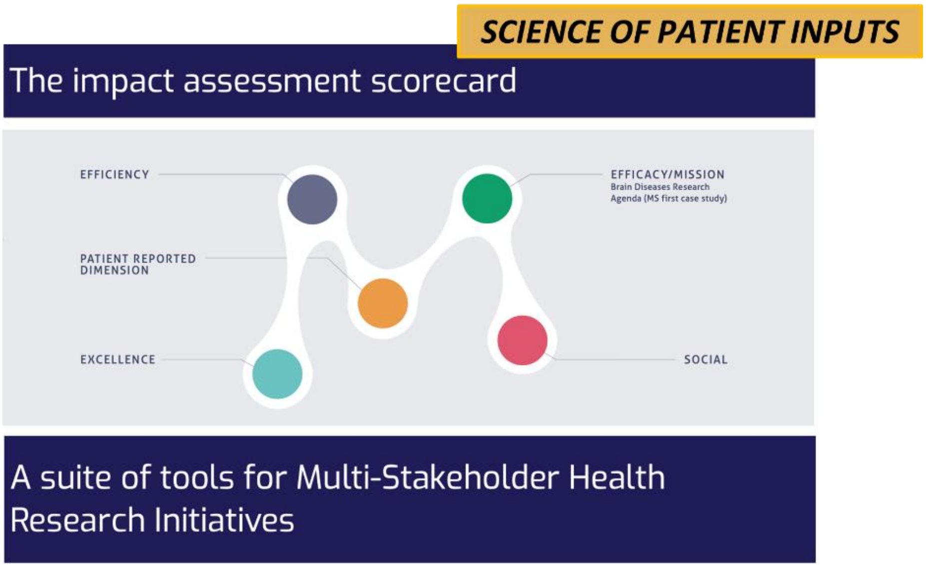

Figure 3. Impact assessment model

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[23] Deliverable D5.4: "MULTI-ACT Governance Model for collaborative initiatives", Jun 2019, https://www.multiact.eu/project-deliverables/

[24] Deliverable D3.6: "MULTI-ACT Impact Assessment Master Scorecard", Nov 2019, https://www.multiact.eu/project-deliverables/

4. CALL TO ACTION FOR EFFECTIVE PATIENT ENGAGEMENT AS A KEY FEATURE OF RESPONSIBLE HEALTH RESEARCH AND INNOVATION

Policy makers and research funders[25], as decision makers in the support of Health R&I initiatives play an important role to foster the uptake of an RRI approach by Health Research Funding and Performing Organisations (RFPOs) that aim to conduct their research in a co-creation and co-accountable approach.

The MULTI-ACT Patient Engagement guidelines[26] can be translated into tangible policy recommendations or requirements for RFPOs seeking financial support. This would foster their uptake of an RRI approach which, in the case of health research, entails the inclusion of the patient perspective throughout the research continuum.

Policy makers can do this in different ways:

1) to require RFPOs to conduct their R&I with a multi-stakeholder and co-accountable approach, taking advantage of the EU-funded MULTI-ACT framework (i.e. guidelines and tools).
2) to require RFPOs of multi-stakeholder research initiatives, seeking to engage with patients, to use or adapt their patient engagement strategies to essential aspects of the MULTI-ACT Patient Engagement guidelines and Roadmap[27]
    a. The establishment and training of the Engagement Coordination Team (ECT) responsible for the integration of the patient experiential knowledge in the research initiative. MULTI-ACT strongly encourages the members of this team to gather patients' stories and information and translate them into experiential knowledge throughout the R&I path. It is also recommended to work with patient organizations which play a pivotal role in enabling the transition from individual to collective patients' experiential knowledge.
    b. The development of the Patient Engagement Plan aligned with the mission/agenda. The Patient Engagement Plan is a managerial tool to support operationalization of Patient

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[25] Example of **Policy Makers**: European Commission, its Directorates and Agencies, National Ministries of Health, National Ministries of Research and Education, Regional and Local Health Authorities, Regional and Local Innovation Authorities; **Research Funding Programmes and Initiatives:** EU funding programmes (e.g. HORIZON EUROPE, EU4HEALTH PROGRAMME, territorial cooperation programmes such as INTERREG, etc...); funding initiatives backed by the EU and the Member States (e.g. AAL, ERANET, JPI, etc.); public-private initiatives (e.g. IMI, EIT Health, etc.), Non-profit organisations, Foundations funding research on neurodegenerative diseases (example: National: FISM, National MS Societies, International: Progressive MS Alliance - PMSA, etc.), Universities, Research Centres, University Hospitals, Healthcare Organisations.

[26] MULTI-ACT Patient Engagement Guidelines, short version v0.1 May 30th 2020, https://www.multiact.eu/project-deliverables/

[27] MULTI-ACT proposes a Patient Engagement Roadmap that consists of four fundamental activities: i) setting up an Engagement Coordination Team with trained figures; ii) selecting the research steps where patient engagement is instrumental to meet the project's objectives and mission; iii) developing an engagement plan for each identified research steps; and iv) selecting the indicators to be used to measure the success and effectiveness of this engagement.

Engagement in research and its structure is designed to comply with minimum requirements established to ensure proper patient engagement:

   i. Select actions of patient engagement that needs to be implemented in order to achieve the vision and mission of the project;
   ii. Define roles and responsibilities of the team that should manage and carry out the implementation of such Patient Engagement actions;
   iii. Design mechanisms to value and acknowledge the experiential knowledge of patients, including the establishment of appropriate recognition of patient contribution, and avoid tokenism;
   iv. Select/design methods to ensure representativeness of the patient community relevant for the mission;
   v. Choose clear and measurable targets (measuring the performance and effectiveness/value - Return on Engagement);
   vi. Present a clear timeline of activities and sustainable budget;

  c. The monitoring and assessment of the plan and its impact on outcomes that matter most to patients.

3) to provide adequate funding to support the essential steps of the patient engagement strategy (as outlined in the above paragraph) in health R&I projects.

4) to recommend the use of metrics to evaluate patient engagement actions in health research initiatives. It is suggested to use a mix of quantitative and qualitative metrics for the performance[28], effectiveness and value of patient engagement in R&I processes. MULTI-ACT has developed metrics to guide multi-stakeholder brain research initiatives applying the MULTI-ACT framework[29].

These requests should be included in all applications for Health Research funding with dedicated questions in the application form such as: “Please define your patient engagement plan?” “Who will form part of your Engagement Coordination Team?” “What metrics will be used to measure performance and effectiveness of patient engagement?”

The EU-funded MULTI-ACT project invites policy makers to endorse its patient engagement guidelines and to take action to foster their adoption in all its funded health R&I projects.

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[28] The MULTI-ACT digital functionality can be used to develop the patient engagement (www.multiact.eu)

[29] See Section “Measure the performance and effectiveness of patient engagement” of MULTI-ACT Patient Engagement Guidelines (page 11), short version v0.1 May 30th 2020 and Deliverable D1.8 https://www.multiact.eu/project-deliverables/

APPENDIX 1 Short version to be circulated to policy makers[30]

The Short version will be circulated to policy makers and R&I funders. A preliminary list of target bodies and institutions to which this call is addressed is presented below.

Policy Makers: European Commission, its Directorates and Agencies, National Ministries of Health, National Ministries of Research and Education, Regional and Local Health Authorities, Regional and Local Innovation Authorities.

Research Funding Programmes and Initiatives: EU funding programmes (e.g. HORIZON EUROPE, EU4HEALTH PROGRAMME, territorial cooperation programmes such as INTERREG, etc...); funding initiatives backed by the EU and the Member States (e.g. AAL, ERANET, JPI, etc.); public-private initiatives (e.g. IMI, EIT Health, etc.).

Research Funding and/or Performing Organisations: Non-profit organisations, Foundations funding research on neurodegenerative diseases (example: National: FISM, National MS Societies, …. International: Progressive MS Alliance (PMSA), …), Universities, Research Centres, University Hospitals, Healthcare Organisations.

1. PROBLEM STATEMENT

1.1 Contextual background

According to a recent consensus document of the H2020 Scientific Panel for Health entitled 'Building the future of health research - Proposal for a European Council for Health Research', **"health, health research and health care has to form a unique and interdependent ecosystem. Healthcare cannot be separated from research", Health and health care are pillars of the social structure, and a public and societal responsibility"**[31].

To close the gap between science and society, the European Commission recommends and actively promotes the policy of Responsible Research and Innovation (RRI). RRI is defined as "an approach that anticipates and assesses potential implications and societal expectations with regard to Research and Innovation (R&I), with the aim to foster the design of inclusive and sustainable research and innovation"[32]. RRI has been highlighted throughout the objectives of the EU Horizon 2020 Programme.

In addition to sound research ethics, a key feature of RRI is the involvement of different societal stakeholders throughout the research process. It implies that societal actors (researchers, citizens, policymakers, business, third sector organisations, etc.) work together during the whole research and innovation process in order to better align both the process and its outcomes with the values, needs and expectations of society.

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[30] The present version will be graphically edited and converted in a publishable version based on the MULTI-ACT look and feel.

[31] A consensus document of the H2020 Scientific Panel for Health / 15.05.2018. The Scientific Panel for Health (SPH) is a science-led expert group based on the provisions of the Horizon 2020 Specific Programme that has been tasked with helping to achieve better health and wellbeing for all. https://ec.europa.eu/programmes/horizon2020/en/h2020-section/scientific-panel-health-sph

[32] https://ec.europa.eu/programmes/horizon2020/en/h2020-section/responsible-research-innovation

In health research particularly, RRI means to put the expectations of patients and society at the heart of research programs. The thinking that underlines patient engagement in RRI argues that excellence, validity, and relevance are connected by engaging patients and society in the research continuum as key stakeholders[33].

It is now more widely recognized that the lived experience of patients, referred as 'patients' experiential knowledge', leads to knowledge that complements the expertise of researchers.

However, the monitoring of the evolution and benefits of RRI has highlighted among others unmet needs in the area of patient engagement.

1.2 Current issues for effective patient engagement

The development of the MULTI-ACT Patient Engagement guidelines followed an evidence-based process. They were driven by a mission-oriented research approach[34] where patients were engaged in research processes and decision making as a key stakeholder category to fulfil the mission.

A key milestone in this process was a landscape analysis of existing patient engagement experiences in brain research[35].

The landscape analysis focused on brain disorders and was performed with different methodological steps: literature review, web research, interviews, surveys and connection with other relevant RRI initiatives. The collection of existing patient engagement procedures, experiences and best practices in R&I and a public consultation were instrumental to develop the MULTI-ACT Patient Engagement guidelines.

The web-search on mapping portals and European repositories found that experiences of patient engagement mainly concern adherence to medical treatments or participation in clinical trials and drug development, in which patients are not engaged from the very beginning of the research with decision making role but consulted for feedback and drug testing.

The literature review found that the majority of studies focus on discussing experiences of engaging patients in healthcare and/or clinical research projects rather than engaging patients in the governance of wider R&I programs. So, patients are very rarely engaged in the governance of R&I according to the dictates of RRI.

The research projects in the health sector appear to have a rather limited repertoire of methods used for engaging patients. The methods are not discussed in terms of their (potential) impact. In

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[33] https://www.rri-tools.eu

[34] Mazzucato M (2018), Mission-Oriented Research & Innovation in the European Union A problem-solving approach to fuel innovation-led growth, EC Directorate-General for Research and Innovation , available at https://ec.europa.eu/info/sites/info/files/mazzucato_report_2018.pdf.

[35] 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/).

conclusion, it was not possible to determine return on engagement[36] of the methods based on the literature.

The landscape analysis demonstrated that the patients as a key stakeholder category is poorly represented in R&I and there is a need for clear and evidence-based methods for guidance on when and how to engage patients and public at all stages of R&I.

The analysis concluded that it was necessary to develop minimum quality criteria for the development, content, and governance of patient engagement.

There is also a need for clear methodologies and metrics to assess the impact and (cost-) effectiveness of patient engagement in R&I as well as clear and constructive communication and professional management of stakeholders.

Take home messages from the landscape analysis were:

- The patients' experiential knowledge should not be confused with scientific/clinical knowledge, as it provides relevant and different insights on R&I[37];
- Patients, placed at the same level and with similar decision-making roles as researchers and other stakeholders, have the potential to maximise the impact of R&I and to assess it according to the results that matter most to them;
- Patients organizations play a pivotal role in enabling the transition from individual to collective patients' experiential knowledge;
- Professional management of all stakeholders is effective in empowering patients' experiential knowledge in R&I, to better prioritize the needs of patients and society.

2. MULTI-ACT PATIENT ENGAGEMENT GUIDELINES

MULTI-ACT stems from the acknowledgement that stakeholder engagement in health research and innovation is an important pathway to achieving impact. It has created a new governance model allowing for the effective cooperation of all relevant stakeholders.

MULTI-ACT aims to fill the gaps identified in the landscape analysis by proposing a roadmap to capture 'experiential knowledge' of patients (figure 1), a knowledge that complements the expertise of researchers and that should be acknowledged and used as a valuable asset for research.

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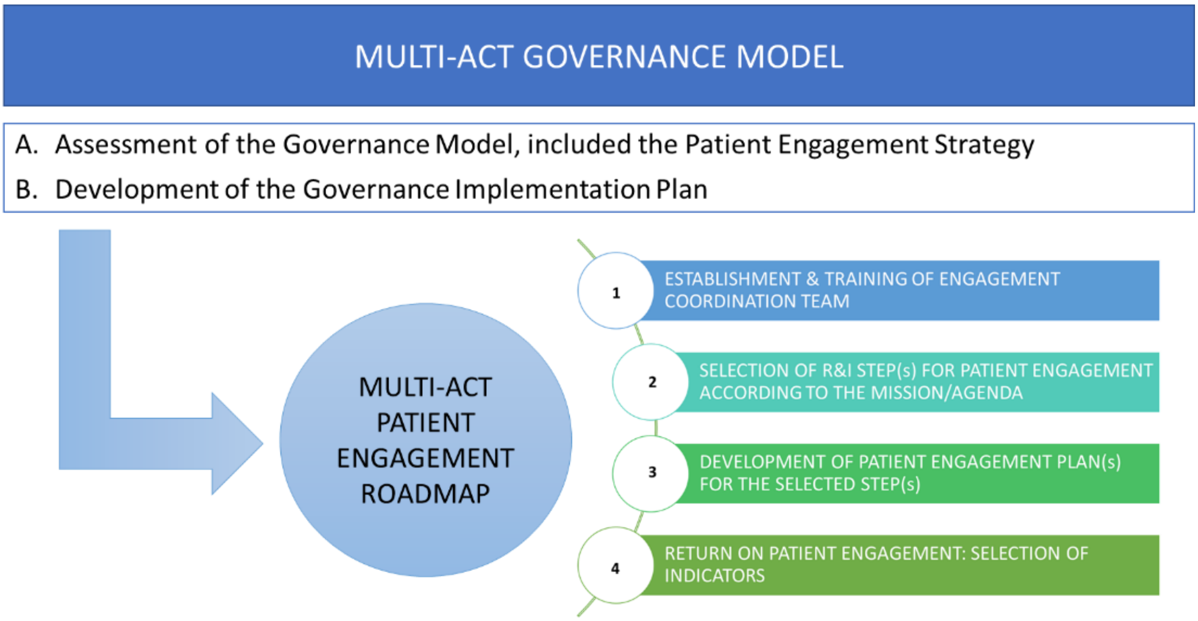
[36] Return on Engagement: the benefit and impact resulting to performing patient engagement in R&I. Evaluating whether engagement adds value for different stakeholder groups can be an effective tool to further support patient engagement and requires the development of metrics to measure the "return on engagement". The MULTI-ACT guidelines include a list of metrics to measure the performance and effectiveness of patient engagement in R&I.

[37] The engagement of people with and affected by the disease as partners with the "experience" of the disease across the continuum of research, development, and care is needed. Therefore, MULTI-ACT is attempting to strengthen the engagement of patients and public that may not have technical knowledge in R&D or regulatory processes ("expert patients" https://www.frontiersin.org/articles/10.3389/fmed.2018.00270/full), but that have the main role to contribute with their experiential knowledge of the disease across the continuum of health research and care.

In fact, the life experience of patients leads to knowledge that complements the expertise of researchers and provides relevant and different insights on R&I which can potentially increase the impact of R&I on the outcomes that matter most to patients.

In MULTI-ACT, the term “patients” refers to the persons with lived experience of the disease, and persons or groups that are affected by the disease, including family members and caregivers.

Figure 1 MULTI-ACT Patient Engagement Roadmap



The MULTI-ACT Patient Engagement guidelines[38] provide a strategy to empower the stakeholder “patients” to be engaged in R&I and to empower all the stakeholders to collaborate and co-create with the “patients”.

The MULTI-ACT Patient Engagement guidelines are one of the three key components of the MULTI-ACT framework alongside the Governance model[39] (figure 2) and the co-accountability impact assessment model[40] (figure 3).

Measuring impact of health research on Patient Reported Dimension will maintain patients engaged as key stakeholder.

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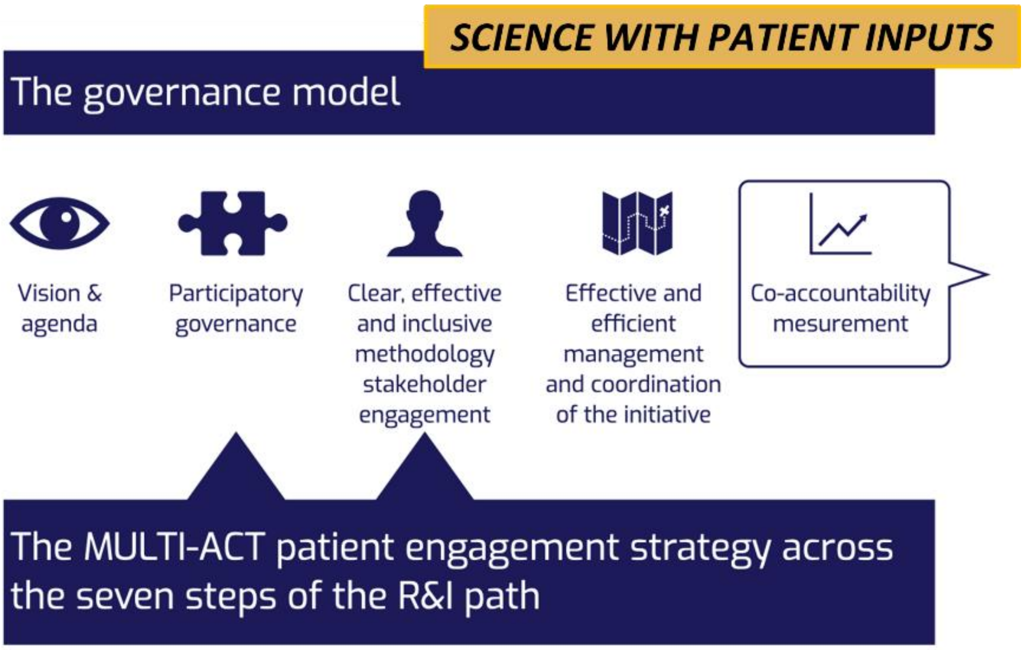
[38] MULTI-ACT Patient Engagement Guidelines, short version v0.1 May 30th 2020, https://www.multiact.eu/project-deliverables/

[39] Deliverable D5.4: “MULTI-ACT Governance Model for collaborative initiatives”, Jun 2019, https://www.multiact.eu/project-deliverables/

[40] Deliverable D3.6: “MULTI-ACT Impact Assessment Master Scorecard”, Nov 2019, https://www.multiact.eu/project-deliverables/

Figure 2: governance model



SCIENCE WITH PATIENT INPUTS

The governance model

Vision & agenda

Participatory governance

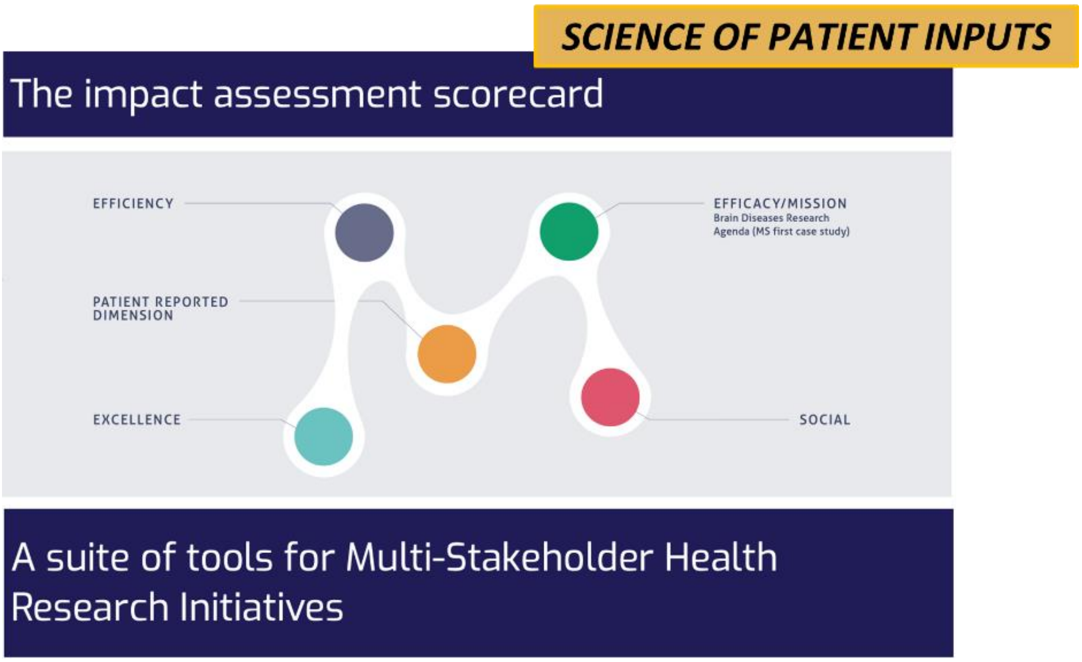
Clear, effective and inclusive methodology stakeholder engagement

Effective and efficient management and coordination of the initiative

Co-accountability mesurement

The MULTI-ACT patient engagement strategy across the seven steps of the R&I path

Figure 3: impact assessment model



SCIENCE OF PATIENT INPUTS

The impact assessment scorecard

A suite of tools for Multi-Stakeholder Health Research Initiatives

3. CALL TO ACTION FOR EFFECTIVE PATIENT ENGAGEMENT AS A KEY FEATURE OF RESPONSIBLE HEALTH RESEARCH AND INNOVATION

Policy makers and research funders[41], as decision makers in the support of Health R&I initiatives play an important role to foster the uptake of an RRI approach by Health Research Funding and Performing Organisations (RFPOs) that aim to conduct their research in a co-creation and co-accountable approach.

The MULTI-ACT Patient Engagement guidelines[42] can be translated into tangible policy recommendations or requirements for RFPOs seeking financial support. This would foster their uptake of an RRI approach which, in the case of health research, entails the inclusion of the patient perspective throughout the research continuum.

Policy makers can do this in different ways:

1) to require RFPOs to conduct their R&I with a multi-stakeholder and co-accountable approach, taking advantage of the EU-funded MULTI-ACT framework (i.e. guidelines and tools).
2) to require RFPOs of multi-stakeholder research initiatives, seeking to engage with patients, to use or adapt their patient engagement strategies to essential aspects of the MULTI-ACT Patient Engagement guidelines and Roadmap[43]
    a. The establishment and training of the Engagement Coordination Team (ECT) responsible for the integration of the patient experiential knowledge in the research initiative. MULTI-ACT strongly encourages the members of this team to gather patients' stories and information and translate them into experiential knowledge throughout the R&I path. It is also recommended to work with patient organizations which play a pivotal role in enabling the transition from individual to collective patients' experiential knowledge.

    b. The development of the Patient Engagement Plan aligned with the mission/agenda. The Patient Engagement Plan is a managerial tool to support operationalization of Patient Engagement in research and its structure is designed to comply with minimum requirements established to ensure proper patient engagement:

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[41] Example of **Policy Makers**: European Commission, its Directorates and Agencies, National Ministries of Health, National Ministries of Research and Education, Regional and Local Health Authorities, Regional and Local Innovation Authorities; **Research Funding Programmes and Initiatives:** EU funding programmes (e.g. HORIZON EUROPE, EU4HEALTH PROGRAMME, territorial cooperation programmes such as INTERREG, etc...); funding initiatives backed by the EU and the Member States (e.g. AAL, ERANET, JPI, etc.); public-private initiatives (e.g. IMI, EIT Health, etc.), Non-profit organisations, Foundations funding research on neurodegenerative diseases (example: National: FISM, National MS Societies, …. International: Progressive MS Alliance (PMSA), etc.), Universities, Research Centres, University Hospitals, Healthcare Organisations.

[42] MULTI-ACT Patient Engagement Guidelines, short version v0.1 May 30th 2020, https://www.multiact.eu/project-deliverables/

[43] MULTI-ACT proposes a Patient Engagement Roadmap that consists of four fundamental activities: i) setting up an Engagement Coordination Team with trained figures; ii) selecting the research steps where patients engagement is instrumental to meet the project's objectives and mission; iii) developing an engagement plan for each identified research steps; and iv) selecting the indicators to be used to measure the success and effectiveness of this engagement.

   i. Select actions of patient engagement that needs to be implemented in order to achieve the vision and mission of the project;
   ii. Define roles and responsibilities of the team that should manage and carry out the implementation of such Patient Engagement actions;
   iii. Design mechanisms to value and acknowledge the experiential knowledge of patients, including the establishment of appropriate recognition of patient contribution, and avoid tokenism;
   iv. Select/design methods to ensure representativeness of the patient community relevant for the mission;
   v. Choose clear and measurable targets (measuring the performance and effectiveness/value - Return on Engagement);
   vi. Present a clear timeline of activities and sustainable budget;

c. The monitoring and assessment of the plan and its impact on outcomes that matter most to patients.

3) To provide adequate funding to support the essential steps of the patient engagement strategy (as outlined in the above paragraph) in health R&I projects.

4) To recommend the use of metrics to evaluate patient engagement actions in health research initiatives. It is suggested to use a mix of quantitative and qualitative metrics for the performance,[44] effectiveness and value of patient engagement in R&I processes. MULTI-ACT has developed metrics to guide multi-stakeholder brain research initiatives applying the MULTI-ACT framework[45].

These requests should be included in all applications for Health Research funding with dedicated questions in the application form such as: "Please define your patient engagement plan." "Who will form part of your Engagement Coordination Team?" "What metrics will be used to measure performance and effectiveness of patient engagement?"

The EU-funded MULTI-ACT project invites policy makers to endorse its patient engagement guidelines and to take action to foster their adoption in all its funded health R&I projects.

Policy makers could provide guidance on how to facilitate and moderate the co-creation process in Health R&I, enabling the concept of RRI. Stakeholders' representativeness in the governance and implementation of R&I will maintain stakeholders' engagement, ensuring endorsement and uptake of research results, making effective the use of public resources' directed to R&I.

MULTI-ACT Patient Engagement guidelines also available on: www.multiact.eu/project-deliverables/

MULTI-ACT Project

- Website: www.multiact.eu

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[44] The MULTI-ACT digital functionality can be used to develop the patient engagement (www.multiact.eu)

[45] See Section "Measure the performance and effectiveness of patient engagement" of MULTI-ACT Patient Engagement Guidelines (page 11), short version v0.1 May 30th 2020 and Deliverable D1.8 https://www.multiact.eu/project-deliverables/

- MULTI-ACT Video: https://www.multiact.eu/multimedia/#multimedia_5

Patient Engagement Guidelines:

- News on MULTI-ACT website: https://bit.ly/36GaqwG
- Video of the MULTI-ACT Patient Engagement guidelines: https://youtu.be/JS1y-5bwO4U
- Video on the partnership between PMSA e MULTI-ACT: https://www.youtube.com/watch?v=rzuABh-jmXY

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