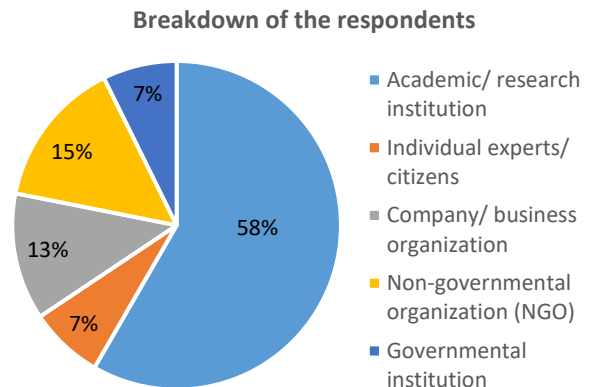


Results of the public consultation on the Strategic Research & Innovation Agenda for the Innovative Health Initiative Joint Undertaking under Horizon Europe

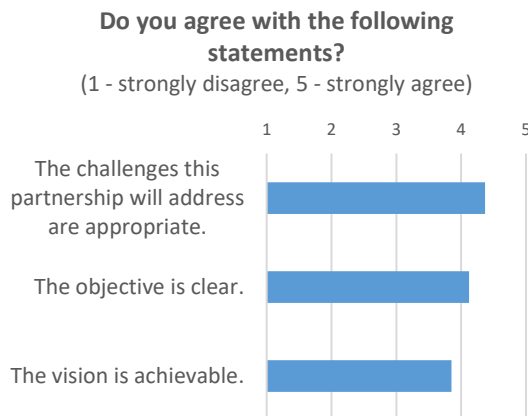
The consultation on the Strategic Research Agenda (SRA) for Innovation in Healthcare collected suggestions and opinions about the goals and the actions stated in the Agenda. The consultation was open from 24 October until 4 December 2019.

Respondents were in total sum 96; 40 out of them opted for an anonymous contribution. Several respondents integrated their submission with further documents.



Outcomes of the consultation

Respondents on average believe that the challenges addressed by this Public Private Partnership are appropriate and that the stated objective of the Strategic Research Agenda is clear. Although lower than for the previous questions, the achievability of the vision meets consensus as well.



See the table in Appendix I for more details.

Concerning any missing challenge, the suggestions collected through the consultation mention the importance of prevention and the close relation between citizen’s health and their lifestyle. Innovation is tackled from the perspective of accessibility and affordability. Education of the health workforce is seen as essential to concretely

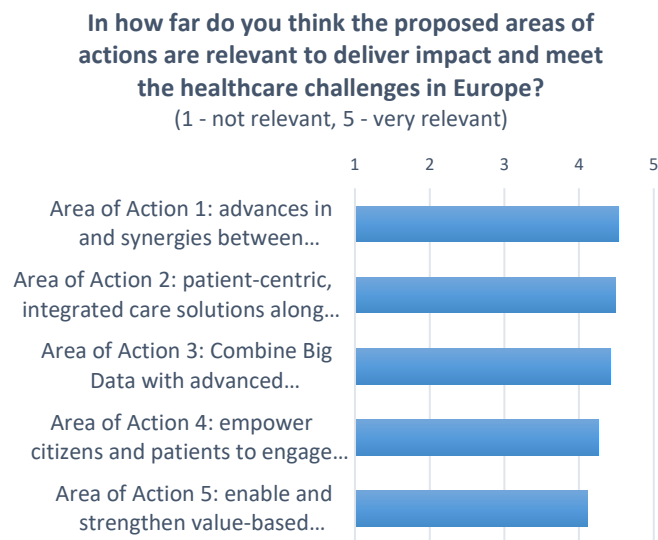
apply innovation.

Ethical challenges in the development of Artificial Intelligence (AI) and support for Health AI start-ups should be addressed. More attention towards the security of patients’ data is required. Regulation is considered a hurdle that must be accounted for and faced.

Respondents also encourage to build upon the legacy of IMI2 and to improve already existing technologies. Collaboration between different actors is considered critical, especially with regard to research institutions and SMEs.

A focus on a number of specific diseases and health issues is suggested ranging from chronic and infectious diseases to AMR to multimorbidities. ATMPs are also mentioned to be included in the SRA. Finally, a value-based approach should consider spillover effects to other sectors of the economy and the impact on carers.

Respondents **consider all the proposed areas of action as relevant** for the successful achievement of the vision.



They propose **integrations to the existing areas** as follows:

- Area of action 1: to give major importance to ICT (software and hardware) innovation and to take into account SMEs which allow for delivering innovation coming from academia to the market. Moreover, AMR and infectious diseases should represent key topics. Technology should be applied to guarantee increased integration in healthcare. More details about the partnership with laboratories are required;
- Areas of action 2 and 3: focus on the importance of integrated healthcare;
- Area of action 4: should be led

(together with area 5) by clinical experts;

- Area of action 5: the value produced should also be relevant to healthcare providers and the sustainability of the action itself raises concerns. A critic is the failure to tackle unmet medical needs through this area.

New areas of action are also proposed:

- A focus on technological diagnostic and treatment methods;
- Collaboration on a global level and a more solid use of/ communication with the European research infrastructure;
- Regulation, together with interoperability and standardization issues;
- Measurement and monitoring activities of the outcomes of new technologies;
- Accessibility and affordability of innovation;
- Support to SMEs;
- Improvement of already-existing technological solutions;
- Inclusion of ‘common-good advocates’ such as academics who would not have conflict of interest.

Respondents agree as well in proposing **initiatives related to the scope of the SRA**. These initiatives mostly concern academia, regional and governmental institutions.

When asked about input for projects to realize under the next Health PPP, respondents suggest both topics and project ideas, most of them related to innovation or key diseases. *Appendix II and III provide a full list of initiatives and projects.*

According to the respondents, on the one hand, key **disease areas** this partnership should focus on include infectious diseases (HIV and AIDS, malaria ...), lifestyle related and chronic diseases (diabetes, obesity, asthma, chronic pain, cancer ...), mental health, neurodegenerative diseases. On the other hand, examples of **unmet medical needs** to consider in the partnership are:

- Integration of pharmacotherapy into multimodal management programs. Implementation of prevention, management and self-management of acute and chronic pain;
- Artificial organs based on stem cell technology and 3D printing of biomaterials that could ease the shortage of organs for transplantation such as heart, liver, kidney;
- Automated connectivity for Systems Medicine applications to healthcare;
- Increased research focus on cardiovascular diseases.

The **indicators suggested** for measuring the impact and performance of the partnership are:

- Quantifiers for the uptake of innovation;
- Indicators related to patients' hospitalization and healthcare continuum (access, diagnosis, treatment, care related);
- Indicators related to the actors involved in the Health PPP (SMEs, research institutions);
- Indicators for the economic impact;
- Indicators for research value.

Appendix IV includes a full list.

Appendix I: Overview of the results for the quantitative answers

Question	Average answer	Share of respondents
<i>Do you agree with the following statements? (1 - strongly disagree, 5 - strongly agree)</i>		
The challenges this partnership will address are appropriate.	4.37	99%
The objective is clear.	4.12	99%
The vision is achievable.	3.85	99%
<i>In how far do you think the proposed areas of actions are relevant to deliver impact and meet the healthcare challenges in Europe? (1 - not relevant, 5 - very relevant)</i>		
Area of Action 1: advances in and synergies between genetics, biology and technology innovations for more precise and effective prevention, diagnosis, treatment and care;	4.53	97%
Area of Action 2: patient-centric, integrated care solutions along the entire healthcare continuum;	4.49	96%
Area of Action 3: Combine Big Data with advanced analytics/artificial intelligence to enable the new integrated healthcare approach;	4.42	97%
Area of Action 4: empower citizens and patients to engage with, manage and improve their health;	4.26	97%
Area of Action 5: enable and strengthen value-based decision making in health and social care systems.	4.12	99%

Appendix II: List of initiatives in the scope of the SRA

Note: repetitions were omitted from the list.

- Fraunhofer SCAI-Bioinformatics
- Regione Marche (IT) as Reference site for EIP on AHA
- IMI call 17 on new chemical tool compounds is important for Area 1
- EIBIR Strategic Research Agenda for Biomedical Imaging
<https://insightsimaging.springeropen.com/articles/10.1186/s13244-019-0684-z>
- ATMP: Valencian Regional Strategy RIS3-FEDER (ES)
- Swiss Personalized Health Network (SPHN)
- “ Le droit des médicaments orphelins en Europe ” (PhD thesis) :
<https://www.theses.fr/2017USPCB179>
- L’université des patients : <https://universitedespatients-sorbonne.fr/>
- Beneluxa initiative “Horizon scanning”: <https://beneluxa.org/horizonscanning>
- Duchenne UK “Project Hercules” : <https://www.duchenneuk.org/project-hercules>
- Banque national de données des maladies rares : <http://www.bndmr.fr/>
- Eurordis, Rare Diseases Europe : <https://www.eurordis.org/content/eurordis-community-advisory-board-cab-programme>
- Eurobiomed : <https://www.eurobiomed.org/>
- NEXTCARE: www.nextcarecat.cat
- Followknee project : <https://followknee.com/>
- Handicap Innovation Territoire : <https://hit-lorient.bzh/>
- European patients forum (EPF)
- European Patients academy (EUPATI)
- Coreva Scientific is engaged in objectively assessing value of medical devices in a number of European hospitals. This includes coming to consensus on "value" and collecting data to aid in its measurement.
- DigiMed Bayern for the Medicine of the Future: <https://www.bio-m.org/en/about-biom/projects/digimed-bayern.html>
- AIFP.cz advisory panel on orphan medicinal products
- HIMSS organization: himss.org
- international interoperability requirements Global Digital Health Partnership: gdhp.org
- From the Finnish Government:
 - Personalized medicine: <https://stm.fi/en/personalized-medicine>
 - Secondary use of health and social data: <https://stm.fi/en/secondary-use-of-health-and-social-data>
 - The Well-being and Health Sector’s Artificial Intelligence and Robotics Programme (Hyteairo): <https://stm.fi/en/the-well-being-and-health-sector-s-artificial-intelligence-and-robotics-programme-hyteairo->
- ECSEL-JU programme: <https://www.ecsel.eu/>
- The University of Glasgow led a UK Government (Department for Business, Energy & Industrial Strategy) Science & Innovation Audit of Precision Medicine (Precision Medicine Innovation in Scotland: Accelerating Productivity Growth for Scotland and the UK) published in March 2019: <http://gla.ac.uk/precisionmedicine/sia>
- SiLA standards in laboratory automation: <https://sila-standard.com/>

- The European Respiratory Society (ERS) Clinical Research Collaboration on Severe Asthma (SHARP) is currently developing an initiative
- Biomedical Research for the 21st Century Collaboration: www.biomed21.org
- The NOBEL project: www.nobel-project.eu
- Radboud AI for Health - Innovation Center for Artificial Intelligence: <https://icai.ai/radboud-ai-for-health>
- OnePlanet Research Center: <https://oneplanetresearch.nl/en/homepage>
- TopFit: <https://www.topfit.life>
- The anDREa consortium: <https://www.andrea-consortium.org>
- ICMS: <https://www.health-holland.com/partnerships/ICMS>
- IMI Pain Care: <https://www.imi-paincare.eu/>
- The European Society of Anaesthesiology (ESA): www.esahq.org
- Research Programmes of the Academy of Finland
 - Molecular Regulatory Networks of Life, <https://www.aka.fi/en/research-and-science-policy/academy-programmes/current-programmes/RLife/>
 - Climate Change and Health (CLIHE), <https://www.aka.fi/en/research-and-science-policy/academy-programmes/current-programmes/environmentalhealth/>
 - Health from Cohorts and Biobanks (COHORT) (2017-2020) <https://www.aka.fi/en/research-and-science-policy/academy-programmes/current-programmes/cohort/>
 - Health from Science TERVA (2018-2020) <https://www.aka.fi/en/research-and-science-policy/academy-programmes/current-programmes/health-from-science/>
 - Personalised Health – From Genes to Society, pHealth (2015–2021) <https://www.aka.fi/en/research-and-science-policy/academy-programmes/current-programmes/phealth/>
- Centres of Excellence (CoE) funded by the Academy of Finland
 - The Centre of Excellence in Body-on Chip Research: <https://www.bodyonchip.fi/>
 - The Centre of Excellence in Research on Ageing and Care
 - The Centre of Excellence in Tumour Genetics Research
- Netherlands Centre for One Health: <https://ncoh.nl/>
- European Lead Factory: www.europeanleadfactory.eu
- The Interagency Pain Research Coordinating Committee of NIH has developed a Federal Pain Research Strategy that stretches across all fields of biomedical research
- German Platform NanoBioMedicine: www.dp-nbm.de
- NOBEL project: www.nobel-project.eu
- The Total Patient Management (TPM) project in Sardegna (Italy)
- Bridging technologies for healthcare innovation : <https://www.flanders.health/>
- Stimulate innovation in the development of immunotherapeutics and vaccines by catalyzing new public-private partnerships: <https://flandersvaccine.be/>
- Virtual institute of Dutch and Belgian public (universities and governments) and private (health foundations and companies) partners that will work together to develop regenerative medicine solutions to health challenges: <https://reqmedxb.com/>
- Asthma UK: <https://www.asthma.org.uk/>
- EC - DG GROW – IPCEI Strategic Forum – Smart Health Strategic Value Chain “Strengthening Strategic Value Chains for a future-ready EU Industry” - report of the

Strategic Forum for Important Projects of Common European Interest:

<https://ec.europa.eu/docsroom/documents/37824>

- European Digital Innovation Hub in Healthcare Robotics: <https://dih-hero.eu>
- eHealth Application Experiment of DIH DIATOMIC: <https://diatomic.eu/index.php/push-experiments/ehealth/>
- Reference regional sites of EIP Active and Healthy Aging
- The French Health Data Hub: www.health-data-hub.fr
- Start-ups digital solutions implemented in medical/social care in Meuse department “e-meuse santé”: www.meuse.fr/popup.php/multimedia/popUpMedia?media=3027
- Regional Innovation Hub specialised in Healthcare (DATALife – Galicia)
- Centre for digital life Norway: www.digitallifenorway.org/gb/
- HUNT - database with information on 120,000 people in Nord-Trøndelag region: <https://www.ntnu.edu/hunt>
- Swiss personalised health network: www.sphn.ch/en.html
- Genes & Health: <http://www.genesandhealth.org/>
- Born in Bradford: <https://borninbradford.nhs.uk/>
- LAMP (Lowering antimicrobial prescribing): <https://www.westyorksrd.nhs.uk/lamp>
- The Dutch Oncode Institute: <https://www.oncode.nl/about/founding-principles>
- EIT Health Vinnova.se. Vision driven health: <https://www.vinnova.se/en/calls-for-proposals/Vision-driven-health/> , <https://www.vinnova.se/en/m/life-science/biologiska-lakemedel/>
- Science for Life Laboratory Sweden Genomic Medicine Sweden (GMS)
- Swelife
- Medtech4Health
- REACT
- Eureka
- Eurostars
- Region Auvergne Rhône-Alpes: https://www.auvergnerhonealpes.fr/uploads/Document/30/496_248_Schema-Regional-d-Enseignement-Superieur-de-Recherche-et-d-Innovation.pdf
- Medical Delta: <https://www.medicaldelta.nl>
- Biohub Slovakia- www.biohub.sk
- Slovacin- <https://slovacin.sk/>
- TRICALS: www.tricals.org
- Project ALS-Care: www.neurodegenerationresearch.eu
- Project MinE: www.projectmine.com
- Basque Health Cluster ETPN
- ECIBC, the European Commission Initiative on Breast Cancer, is operated by the EC’s Joint Research Centre and DG SANTE: <https://healthcare-quality.jrc.ec.europa.eu/discover-ecibc>
- IPAAC, the innovative partnership for action against cancer, develops innovative approaches to advances in cancer control and the ESR is involved in “Work Package 5 – Cancer Prevention”: <https://www.ipaac.eu/>
- Slovak AI

Appendix III: Projects

Note: repetitions were omitted from the list.

List of topics for projects

- ATMP, exosomes
- Integrated care for disabled people
- Patients' data collection, dissemination, delivery, health management
- Metabolomics to study antibiotic resistance and antibiotic action on pathogens
- Metabolomics to study bacterial communication in complex microbiomes
- Ageing
- Influence of infections on the development of neurodegenerative diseases.
- Energy topics for smart medical device
- AMR
- A strategic roadmap for scientific progress
- Epigenetic determinants of steroid resistant wheeze in infants and children
- New supply chains for integrated-technology health solutions
- Patient-driven ideation on future-proof prevention, treatment or healthcare concepts
- Regenerative medicine/3D printing: replacing lymph nodes after cancer treatment to avoid lymphedema
- Scientific evidence based research into effect of music and other environmental stimuli on mental health patients
- Large-scale RWE, gamification citizen science projects on disease emergence and progression of not-well understood diseases (e.g. Parkinson's)
- Incentives for publication of negative clinical or RWE trial results
- P4 medicine (predictive, preventive, personalized, participatory)
- Role of the European medical registries, standardization and interoperability
- Co-morbidities
- SMEs
- Advanced manufacturing
- Diseases in the developing world
- Non-invasive measurement of biomarkers.
- Drug reformulation employing nanotechnologies (in the field of antibiotics for instance).
- Robotics solutions, especially social robots for ageing and care.

List of project ideas

- How European Union can improve the affordability of innovations
- Expanding on or building on the Human Functional Genomics Project, a collaboration of Radboud university medical center, University Medical Center Groningen and University of Bonn, aiming at identifying the consequences of genetic variation in human DNA and the complex colonization with microbial communities (microbiome) have on the physiological processes in the human body with a focus on the immune system in health and human diseases (<http://humanfunctionalgenomics.org/site/>).

- Standardizing ethics considerations and data-protection protocols across frontiers
- Meetings between payers and companies and patient advocates to discuss affordable access
- Apply new technologies for the generation of novel knowledge on molecular mechanisms of multimorbidity clustering and its transfer into innovative healthcare services to generate real world evidence
- A new paradigm of treatable mechanisms for asthma. Problem: For chronic diseases like asthma, most guidelines and diagnostic approaches rely on symptoms and clinical measurements. On their own symptoms and clinical measurements are not adequate for stratifying disease populations. The use of multi-omic molecular profiling to identify molecular mechanisms is now at a stage where there is proof of concept that it improves the stratification of disease populations while identifying cross disease mechanistic pathways. It is also clear that there is an overlap of mechanistic pathways between different diseases. Development of new therapies that address mechanisms common to multiple diseases would be an efficient way to meet unmet needs and co-morbidities in chronic diseases. In addition to treating clinically measurable traits of a disease, more emphasis needs to be placed on treatable mechanisms. The validation of a treatable mechanism strategy, however requires a broad interdisciplinary effort. Project description: The aim of this project is to validate the use of multi-omic molecular profiling as a new paradigm to improve clinical management and to accelerate the pace of new therapy development in asthma. Datasets that have the most potential to meet unmet needs and to bridge across multiple conditions will be used to tailor prospective clinical studies to validate the treatable mechanism paradigm. The validation effort will unfold as a series of rapid-cycle testing in which molecular profiles are used to detect changes in underlying mechanisms and to analyse the impact of targeted interventions and perturbations. The concept of treatable mechanisms will be translated into more patient-friendly non-invasive measurements using MedTech devices to capture samples and measure available signals such as volatile organic compounds in breath, non-invasive sampling of the distal airways, the analysis of biological signals in accessible bodily fluids, as well as artificial intelligence enhanced imaging will be included. Outcome: The validation of a 'treatable mechanisms' paradigm in this project will accelerate the pace of new therapy development and enable more precise clinical management. It will also open up new avenues for cross disease therapies that are likely to be more cost-effective. This will also enable the use of mechanistic insights as part of digitally integrated care.
- Title: Improving the selection of steroid-resistant asthma patients for treatment with expensive biological therapies. Problem: A large proportion of patients with asthma do not or do poorly respond to standard steroid therapy, and suffer from uncontrolled, severe disease. Despite the advent of biological therapies targeting individual inflammatory pathways, which has caused significant improvements in disease control, the success rates of these therapies fall short to 100% reduce acute asthma exacerbations which present the biggest challenge for people with asthma and incur high costs. Various programmes, including the EU-funded Innovative Medicines Initiative U-BIOPRED, have developed multiple 'omics platforms to a) better understand the mechanisms of severe asthma and b) identify predictive and prognostic biomarkers and stratify asthma. The time is ripe and there is a strong need to

validate the phenotypes and endotypes of asthma in real-life studies in which different biological agents are used as part of standard care. Project description: Ongoing efforts to integrate severe asthma registries under a common data model will be extended, allowing data collection from thousands of patients with steroid resistant severe asthma across Europe. These data will be combined with easy to apply biomarker platforms (breathomics, blood transcriptomics and urinomics - a combination of urine eicosanoids and proteomics) that have been recently shown to be predictive of a good clinical response to one of the biologics. These observations will be validated prospectively and also tested for other (novel) biologics, allowing comparisons between biologics in respect of both mechanisms of action and predictive biomarkers. The aim would be to rapidly accumulate evidence for the most effective asthma management strategies in steroid resistant patients by using existing real-world data under the guidance of biomarkers. The additional economic benefit for Europe would be the creation of point of care tests developed by European companies. Outcomes: Generation of real-world data analyses that would support decision making by clinicians for precision management in patients with severe steroid resistant asthma, with additional benefit for public health policy and harmonization of care. On a broader level this project would define the processes and best practices for the rapid generation and agreement on evidence after the introduction of new therapies for any disease.

- Development of non-invasive imaging tools for personalised development and monitoring of novel cell and gene therapies in solid tumour patients [Action Area 1]
- Development and implementation of disease-specific multiomic profiles for personalised medicine [Action Area 2].
- Identification and validation of biomarkers to optimise pediatric patient management [Action Area 1]
- The University of Glasgow is leading an NHS-University-Industry consortium to develop a Living Laboratory at the UK's largest hospital (the Queen Elizabeth University Hospital in Glasgow), which will address the barriers to adoption of precision medicine in a real world clinical setting.
- Expert group of EUHealthPPP stakeholders and regulators at national and European level tasked to discuss and inform each other on how to reduce regulatory fragmentation in the health field, how to tackle innovative cross-sectoral products and the use of ""Big Data"" advance/analytics and AA/AI, etc.
- Develop ICT tools, data-generating wearables and devices, POCs, IT infrastructure to enable telemedicine for the homecare and post-acute care sector
- Support multidisciplinary approaches to specifically tackle complex diseases, e.g. metabolic syndrome
- Develop novel solutions that help de-risking investment in R&I&D to fight against rare diseases / infectious diseases, e.g. HIV, measles.
- Develop the most efficient/innovative medical care for the poorest part of the EU population with precise case studies, e.g. vaccines.
- Develop platforms and processes to engage citizens in a positive way to improve efficiency of preventive medicine

- Continuation of AMR Accelerator programme, Pillar B: Tuberculosis Drug Development Network (TBDDN), in order to address the innovation gap in the discovery and development of a novel drug regimen for all forms of TB. TB Alliance collaborates as an associated partner on the project
- Continuation of the current IMI's Integrated Research Platform, which will convene a broad consortium of stakeholders to develop a reusable, modular approach for the design and execution of patient-centred platform trials. TB Alliance collaborates as an associated partner on the project.
- Harness the potential of e-Health (including m-health) in pain along the continuum of prevention, screening, diagnosis and care
- Contribute to the development screening tools for primary care use that facilitate differential diagnosis of different types of chronic pain (nociceptive, neuropathic, nociplastic) and allow risk-stratification of patients with common chronic conditions with respect to development and prevention of chronic secondary pain conditions (diabetes, rheumatoid arthritis, osteoarthritis, inflammatory bowel disorders, chronic neuropathic pain).
- Integrate efforts with implementation of WHO ICD- 11 codes on chronic pain and WHO (ICF: international classification of functioning, disability and health).
- Support research on the evaluation of electronic tools (e.g. digitalized patient diaries) and telemedicine for communication among healthcare professionals and between healthcare professionals and patients.
- Support the development of web and mobile platforms for continuous medical education on pain, including guideline development and guideline support in pain medicine.
- Initiate funding for development of mobile health (m-health) technologies for patient empowerment and self-care in multimodal pain treatment.
- A "Big Data for Perioperative Patient Safety" project would enable a massive step forward in identifying risks, developing optimisation strategies, evaluating new procedures, technologies and medications, measuring and monitoring postoperative mortality and morbidity, ultimately preventing harm and improving outcomes for patients and healthcare systems.
- Biologics Factory: Build a consortium between leading biotechnology companies and academia under guidance of experienced partnership management organization to translate academic research into high quality therapeutic biological product.
- Project for area of action 1: Novel clinical trial methodologies for measuring efficacy of interventions to prevent or treat chronic neuropathic and musculoskeletal pain in cancer survivors, using innovative biomarkers, compound patient-reported endpoints and advanced systems physiology modelling.
- Project for area of action 2: Patient-centric integrated care models for prevention of chronic pain after surgeries or accidents, using databases and registries and integrated disease management algorithms.
- Project for area of action 3: Rapid implementation of ICD-11 and ICF, the first WHO classification instruments ever that cover chronic primary and secondary pain syndromes and their functional implications for participation in daily life. The API interfaces of ICD-11 will help the European IT industry to deliver interoperable data standards to provide real world data for making evidence-based policy decisions in healthcare. Chronic pain is the single most prominent cause of years lived with disabilities (2015 global burden of disease report).

- Project for area of action 4: Education and empowerment of citizens with chronic pain or headaches to self-manage their medical conditions and their lives. Non-medical interventions and lifestyle changes have already been proven to be effective against chronic pain, but are not yet used widely because of lack of infrastructure.
- Project for area of action 5: Standardisation of assessment of pain severity (intensity, distress, functional impairment) as a quality indicator for health care. (Absence of) pain is a recognized component in all quality of life scales. It is a patient reported outcome that should be part of all HTA assessments (cancer, cardiovascular, neurological, musculoskeletal health etc.). This standard needs to be developed in a multi-stakeholder environment. The Innovative Medicines Initiative (IMI) provides multiple examples how this can be initiated, but so far it lacked follow-up instruments for implementation into health care.
- Area 1 and 2: Psychiatric diseases such as depression are on the rise. Sensors or monitoring devices to analyse changes in speech, movement or behaviour over time with artificial intelligence will enable regulation of medication in real time or intervention of healthcare providers to avoid dramatic outbreaks up to the level of suicide.
- Automation and robotics applied to the development and dissemination of cellular therapy (cell harvesting, banking and manipulation without the variability of using human hands) to overcome the unmet need of the implementation of standardized GCP procedures for cellular therapy preparations (gene, immune, stem cell and probiotic therapies).
- Long term monitoring and drug delivery through wearable systems and flexible patches.
- Preventing and monitoring multimorbidities associated with metabolic and/or chronic diseases (e.g. obesity, diabetes, heart failure, COPD...).
- Integrating stress/emotion measurement in patient outcomes reporting.
- Point of care bio analytics from capillary blood samples (or from unusual solid samples such as faeces). Integrated approaches for rehabilitation after stroke and measurement of outcomes.
- Blood profile measurements along the health trajectory of patients to prevent, predict, treat early diseases shaped by genetic background, life-style, behavioural and environmental circumstances and conditions that individuals experience throughout life.
- Promote and implement projects investigating healthcare solutions (drugs, antimicrobials) with no detrimental or reduced impact on patients microbiome (gut and others)
- An educational base for lifestyle decisions- with help of AI to collect scientifically- based evidence on supporting healthy lifestyle from results of EU projects
- Laboratory-based analysis of foods (e.g. are any vitamins preserved in pasteurised meals? Is cow milk unhealthy for our gastrointestinal tract? etc., questions based on citizen's questions) and material, and research regarding physical activities and nutritional interventions (e.g. omission of certain food groups, protein-based diets, intermittent starvation, effect of food supplements, etc.)
- Harmonised unit cost libraries and resource use information based on the methods currently being developed in the PECUNIA project are fully established and eventually deployed across many countries and sectors to allow proper comparable value assessment in future HTAs/modelling studies/economic evaluations.
- Possible projects to carry out in partnership with the EFS:
 - Develop Car-T technologies (Patent application pending), Innovative medicine products)
 - Develop IPS/ differentiation to others cells type

- Address the blood chain delivery problem (Labile blood product delivery forecast)
- POC Clinique related to use Algorithm for Apherisis Monitoring and Prescription Assistance in Sickle Cell Patients): Exploit better the data.
- Rapid diagnostic test for genotyping based on specific and visual detection of Single-Nucleotid Polymorphism (Patent application pending)
- Use of TLR4 antagonist the treatment of multiple myeloma (Patent application pending)
- Scaled-up research on the development and implementation of image analysis tools along the care pathway in an effort to enable a shift to personalised delivery of care

Appendix IV: List of indicators

Note: repetitions were omitted from the list.

- Uptake of new technologies and medicines throughout EU: changes in clinical guidelines for physicians and parameters such as time-to-diagnosis and time-to-cure, which should increase.
- Number of partners, their distribution in the map and their type (according to quadruple helical classification).
- Number of nosocomial disease, number of patient associations who participate to the debate, number of patient with strong disease who come back to school/ job/ life without (or few) medical assistance, number (en Mbit) of data connected and interoperable, fluidity of the transfert of the medical information between every medical place where a patient goes (doctor, hospital, ...), number of days/ peoples affected by an infectious disease like flu.
- a quantitative measure of success for an innovative procedure could be how many different societal settings it is used in.
- 1. Number of unmet needs addressed with clear prioritisation of the PPP initiatives; 2. Presence of patient representatives and civil society in all strategic decision making with full transparency ; 3. Avoiding financialisation (*sic*) of the projects and aim for reduction of the cost for R&D for all stakeholders; 4. Access granted to all the target population for projects supported; 5. Reduction of the delay for access compared to usual projects; 6. Improvements in the healthcare pathway and quality of life from a patient perspective; 7. Fair remuneration of the achievements done and sustainable investments for R&D to all stakeholders involved with measurement of the health impact of actions in term of added value from a patient perspective.
- Number of research and innovation actions participated by the partnership.
- Number of SMEs involved in projects.
- Cost savings for payors IP generated sales in Europe # patients reached.
- Maybe include KPI's that also look at the long-term impact?
- Lean Six Sigma, various advanced project management tools and some not complicated, but useful diagrams for system thinking in Healthcare (Causal Loop diagram, stock and flow diagram etc). And some tools for dynamic simulations and predictions.
- Sustainability Plan must be part of any partnership to ensure good governance, clarify ownership and ensure the sustainability and affordability of project results and project data.
- Impact should be measured on movement through the value chain.
- Number of new therapeutic approaches reimbursed across Europa Delay between authorization and adoption of new therapies (as short as possible)
- Measures for the economic impact:
 - Financial investment from partners
 - The diversity of multi-sector partnerships, allowing sharing and pooling of expertise from different sectors to offer new solutions to persistent problems
 - Partnerships could lead to the creation of stronger, sustainable networks sharing expertise and resource between sectors to drive ongoing economic growth
 - Measurement of risk reduction for each stakeholder by undertaking a partnership

- Number of patents and sustainable startups (or spin-offs) created
- Value gained by SMEs through access to multi-stakeholder projects, in which they would otherwise lack the resource to partake.
- Measures for research value:
 - Contribution of datasets, knowledge/analysis or other assets to cross-sector medical innovation, including drug and medtech product pipeline development
 - Data integration is designed to be multi-purpose, i.e. for medical research, medtech development, inclusion in the clinical care pathway as part of learning health systems and able to provide a platform for real-world trials
 - Development of new pragmatic trial platforms that can reduce the cost of research
 - Application of technology in one sector (such as AI) that can rapidly advance development in another sector. For example, with new understanding of mechanisms of a disease, AI could more easily identify existing compounds that have value
 - Development of new pre-clinical models that can better determine value of basic science
 - The value of open source data and knowledge that is available to the wider public.
- Measures for innovation:
 - Creation of new, frictionless health journeys, characterised by a marked reduction in time from diagnosis to personalised intervention, treatment and management
 - The use of vast, integrated datasets to create highly-detailed mapping of the needs and behaviours of people to drive better medical innovation, ensuring new products are designed in line with user needs and thus more likely to be accepted
 - Reduced time off school and work, as a result of better disease management
 - Increase in the cost-effectiveness of healthcare, including reduction in hospital admissions, repeat admissions and length of stay.
- Would be good to include measures of innovative suppliers e.g. new companies formed, investment attracted for the innovation in business models in action area 5
- Proposal for indicator for area of action 1: Production and validation of an algorithm for precision management in patients with a complex chronic disease like asthma
- Quantitative indicators with focus on SMEs:
 - Number of SMEs helped / involved in the PPP actions every year
 - Number of new HealthTech SMEs created in Europe
 - Leverage of private fund raising for SMEs supported by the PPP Improved investment and translation of RID in Healthcare
 - Increased number of patents from academia and RTOs through interaction with industry
 - Increased % of sales invested in R&I&D by medical industries
 - New products & solutions available thanks to the PPP
 - Number of successful cross-sectoral projects
 - Number of new diagnostics / drugs / medical devices clinically developed through projects funded by the PPP
 - Number of healthcare solutions for diseases with no treatment developed during the PPP
 - Number of regulatory approved cross-sectoral/multifunctional/multidisciplinary healthcare solutions

- Drop in number of deaths by sepsis (for instance) every year in EU following a dedicated program within the PPP
- Shorter hospitalization of patients with better recovery of patients on selected diseases as result of PPP solutions/results
- Impact on medical outcomes
- Improvement of benefit/risk of selected drugs through smart medical delivery
- Improve clinical outcomes rate in clinical trials through rational design & personalised medicine for new drugs / medical devices Socio-economic impact
- Decrease cost of innovative cancer treatments using new modes of action (e.g. radioenhancers using nanoparticles)
- Increase in job creation in all domains of the Healthcare sector
- Number of employees trained for / acquiring new skills required by Digital Health solutions
- Increase in health insurance coverage of EU citizens
- Decrease the debt of healthcare systems in Europe Take-up of the strategy
- Number of downloads/endorsements of the guidelines and strategic documents provided by the PPP
- Qualitative criteria with focus on SMEs:
 - Uptake of multi-technology medical solutions by industry / clinicians / healthcare systems
 - Better, cheaper, faster access to clinical testing of emerging medical technologies (nanotechnologies, robotics, photonics, advanced materials, digital health)
 - Streamlined regulatory pathways for complex medical devices
 - Improved quality of life for patients with chronic diseases (diabetes, cardiovascular diseases, neurodegenerative diseases, etc.)
 - Improvement of quality & cost efficiency of care in Hospitals when using the value-based procurement methods
 - Impact of Digital Health on the quality of the relation patient / medical staff (less burden for repetitive tasks done by I.A. / more time for personalised care and high-level tasks)
 - Increased awareness of prevention in the EU general public on selected diseases
 - Access to best high-tech medical care in poorest EU countries
- The number of products developed with the support of EU funding and interim milestones of the number of products successfully brought to next stages of preclinical and clinical development.
- Contribute to the implementation of WHO ICD 11 and ICF codes as key step towards facilitate patient management through standardization using these WHO classifications (now available in digital form) and promoting patient-oriented pain management by employing the ICF. This indicators are not only relevant to understanding and treating pain but can also be applied to other areas that were expanded or updated within WHO ICD 11 e.g. mental health, thus this could be an indicator of interest to public and private stakeholders involved in the PPP beyond the pain area.
- Asthma outcomes: admissions/deaths; time off work or out of school; cost of illness
- PKI specifically related to the impact of the patients involvement.
- For patients centered studies in medical practice:

- Availability or not of objective workflow traceability proofs (e.g. direct automated tracking);
- Time to diagnosis from presentation;
- Time to therapy start after diagnosis;
- Number/rate of side effects/complications;
- Costs per correct diagnosis (overall costs for diagnosis in risk patients/number of cases diagnosed);
- Costs per recovery or therapy success (overall costs/number of therapy success).
- For Research Projects:
 - Cost per single point impact factor of publication (Overall funds/overall IF of all publications derived from the project);
 - Cost per single patent (Overall funds /number of patents derived).
- Number of SMEs involved in projects
- Number of RTOs involved in projects
- Research infrastructures, pilot lines, testing facilities used by projects
- Number of spin-offs created 3 years after the end of a project
- Implementation of solutions coming from projects in real settings (clinics, home, social care...)
- Time-to-market of developed solutions
- Number of healthcare systems that uptake developed health innovations
- Healthcare costs saved by developed innovations
- Reduction of the incidence of steroid-resistant disease
- Reduction in the number of exacerbations
- Reduction in number of asthma-related deaths
- Reduction in asthma-related healthcare costs
- Reduction in DALY
- Workshops to enable the exchange of knowledge across industries and countries.
- Qualitative indicators to measure the impact may be new drug targets, promising biomarkers and diagnostic tools. Quantitative indicators to measure impact are the necessary multi-stakeholder approach while addressing a research unmet need.
- Projects funded under the Partnership should be asked to report on how they contribute to achieving SDG3, reporting separately societal impacts, in the form of decreased disease burden and more affordable, accessible health care solutions; and economic impacts (e.g. jobs created).
- Improving the quality of life of people living with ALS, and developing a cure for ALS. Quantitative indicator: reducing the EU mortality number caused by ALS. Qualitative indicator: measuring improvement of the quality of life of people living with ALS.
- Patents, publications, licenses, product commercialisation, social benefits, working changes
- Value based approaches. Patient outcomes on top. Amount of technology really adopted
- The inclusion of 'common-good advocates'

**Appendix V:
Respondents to
consultation**

*Note: respondents who
wanted to remain
anonymous were
removed from the list.*

Bradley English
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18 June 2021

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Rhodri Saunders
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Linktomedicine

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European Hematology
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Jukka Lähesmaa
Ministry of Social Affairs
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Usman Khan
European Patients'
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Scott Wagers
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Platform on
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medical center

Willo Brock
TB Alliance

Jorg Janssen
Lygature

Andrew Bush
Imperial College and
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Loredana Simulescu
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