

Topic: Health Outcomes Observatories – empower patients with tools to measure their outcomes in a standardised manner creating transparency of health outcomes

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Topic details

Action type	Research and Innovation Action (RIA)
Submission and evaluation process	2 stages

Specific challenges to be addressed

Patient outcomes and overall care could be improved through systematic capture of the patient voice and perspective:

Current conceptualisations and measures of disease and clinically relevant disease outcomes have generally been developed from the perspective of the clinician and often fail to completely capture the totality of the disability, the symptoms of the disease and the impact on a patient's health-related quality of life (HRQOL) from the patient's perspective. Important patient-to-patient variations in disease presentation and symptomology may also be lost in the effort to develop a generalisable framework for the disease.

It is important to complement existing clinical outcome measurements with patient-generated measures of disease and HRQOL to ensure that the patient perspective of disease and the impact of healthcare interventions are more completely captured and that disease heterogeneity is better understood.

In order to achieve this, it is essential to provide patients with tools that have the ability to better capture the entirety of the impact of a disease and treatments (e.g. signs, symptoms, tolerability), allowing them to document their disease more completely and in a structured manner. To be effective, these tools should be built on the basis of accepted standards, developed in partnership with all relevant stakeholders and accepted and integrated into the existing healthcare ecosystem.

A reward system that truly focuses on value requires measurement and transparency of patient outcomes:

Disease registries have already been established in a wide range of diseases. However, these registries tend to measure a non-standardised set of outcomes, are rarely interoperable, focus on clinical measurements, and have varying terms and conditions for access to the data captured. As a result, they often fall short from providing sufficient transparency of patient outcomes in specific diseases to inform scientific and policy decisions.

At the level of the individual patient, the data thereby generated, once structured and subjected to a degree of standardisation, will enable patients to have more productive interactions with their healthcare provider. At the level of the healthcare system, this data will allow a systematic measurement of health outcomes and the possibility to set up a reward system based on value – which can be defined as the level of health outcomes achieved for a given cost.

There is a lack of models for capturing and managing patient-reported health data in an ethical and sustainable way:

Structured health data is invaluable for all stakeholders, from the individual patient, health care professionals (HCPs), the life science industry, policy makers to the patient advocacy groups. There have been a few successful examples of approaches to integrate patient-reported health data into clinical care. In an era of greater focus on the patient, it is thus critical for a society that patient-reported health data is captured and managed in an ethical manner ensuring broad and appropriate access while safeguarding patients' privacy and building high levels of trust.

Need and opportunity for public-private collaborative research

Despite rapid advances in medical science and a revolution in health technology, the lack of standardisation and integration of data remains an obstacle to fully realising the promised benefits of the digital revolution¹. Measurement methodologies and outcome standards need to be endorsed by those both generating the data and those using the data and be part of the broader healthcare ecosystem in order to be trusted and accepted. The complexity of the challenges is such, that it requires action that is collective, innovative and nurtured in an environment where sensitive information can be shared securely:

- Patient associations need to engage actively to develop tools and approaches, and to build trust and patient engagement;
- Regulatory authorities need to ensure acceptability of patient-generated data for identification of novel endpoints for the development of new products;
- Privacy and legal experts need to set up the appropriate governance models, consent forms and access terms in order to allow data sharing, ensure trust and, therefore, support sustainability;
- Life sciences companies are critical, not only for bringing in expertise, commitment to long-term research, innovation and evidence generation in the disease areas, but also for providing funding and ensuring that the model can be made sustainable over the long term;
- SMEs and other innovators such as digital companies need to be involved to develop the appropriate tools and technologies;
- Public sector experts including medical experts, ethicists, biostatisticians and researchers are required to identify or develop the appropriate measurements and the right methodologies for capturing and analysing the data;
- Data custodians and data management experts are also essential.

Scope

The goals of this topic are:

¹ As acknowledged by the OECD in their paper: Fujisawa, R. and N. Klazinga (2017), "Measuring patient experiences (PREMS): Progress made by the OECD and its member countries between 2006 and 2016", OECD Health Working Papers, No. 102, OECD Publishing, Paris, <https://doi.org/10.1787/893a07d2-en>

1. Identify appropriate standards for capturing the patient perspective when measuring health outcomes and obtain the support of these standards by the relevant stakeholders. Where appropriate the partners will give preference to standards already being developed (e.g. International Consortium for Health Outcomes Measurement - ICHOM) and will follow the Observational Medical Outcomes Partnership Common Data Model (OMOP CDM) developed through Observational Health Data Sciences and Informatics (OHDSI);
2. Implement appropriate technology solutions (including adopting existing technology where appropriate) that would allow individual patients to record and measure their outcomes according to these standards and use the information for a more structured dialogue with their HCPs;
3. Establish the appropriate platform to collect, process and manage data in the best interest of patients, patient organisations, health authorities, healthcare professionals, the research community and health care payers, and in compliance with General Data Protection Regulation (GDPR) and other relevant rules and regulations;
4. Create a sustainable and ethical model for the continuous collection of data and an appropriate model for providing access to the anonymised or aggregated data to researchers with a legitimate interest in analysing them.

These goals can be achieved through the creation of a consortium whose mission will be to establish Health Outcomes Observatories in three selected disease areas, collecting health data in (at least) three different European countries² for each disease area.

The Observatories should be designed according to the following principles:

- Full integration within the respective countries' health care systems;
- Consistency in design across Observatories;
- A sustainable model for the Observatories;
- Robust patient consent and engagement;
- Standardisation and interoperability across countries;

The disease areas selected are:

- Diabetes Type 1 and Type 2;
- Inflammatory Bowel Disease (IBD);
- Cancer (side effects of chemotherapy and immuno-oncology).

Criteria considered for this selection were: (a) their prevalence in the European population; (b) their chronic and progressive nature; (c) their significant impact in patients' quality of life; (d) their compatibility with patients' digital literacy; (e) the patients' sufficient autonomy and motivation to become engaged in self-management of their disease; and (f) the investment in novel medicines and disease management tools for these diseases by EFPIA members and their Associated Partners.

Expected key deliverables

² European Union and H2020 Associated countries

The overall aim is the creation and operation of Observatories in (at least) the three disease areas identified collecting health data in (at least) three different countries. The deliverables from the project funded under this topic would all be made public and a key objective is to set the Observatories up on a sustainable basis.

To achieve this, the applicants will have to focus on the following deliverables:

- An appropriate governance and sustainability model for the Observatories in three different countries that ensures broad societal acceptance of the model and inclusion in the respective national health ecosystem, and develops revenue streams to fund the continued operation of the Observatories beyond the life of the initial project term;
- All legal and ethical analysis required to ensure appropriate consents for data collection, data management and access terms and conditions;
- The legal set up and operation of the Observatories, sustainable beyond the life of the initial project term;
- The design and set up of the appropriate infrastructure leveraging to the extent possible on existing technological solutions that would allow the collection of the patient-generated data using an accepted common data model (e.g. OMOP CDM);
- The design of a methodology for identifying the appropriate measurements of outcomes for respective diseases taking into consideration the need to also ensure broad stakeholder acceptability of these measurements;
- The identification of the appropriate measurements of outcomes for the focus diseases of this project and the creation of an adequate digital tool leveraging as much as possible existing solutions;
- The launch of the respective digital tools;
- The publication of annual reports after the third year comparing health outcomes in the three countries and identifying lessons learned and opportunities for improvement.

For the three specific disease areas, the work will focus on the following deliverables:

- Identification and validation of key outcome measures to inform health economic evaluations in the disease area;
- Analysis of patient-generated data in combination with electronic health records by means of advanced state of the art analysis techniques to stratify people with the condition to improve the understanding of patient subgroups and determine ideal levels of care;
- A digital decision-making system based on the stratification as above to allow personalised treatment.

Expected impact

Applicants should describe how the outputs of the project will contribute to the following impacts and include baseline, targets and metrics to measure impact:

- Enable **individual patients** to:
 - Receive close to real-time information on their disease status;
 - Hold more informed discussions with healthcare professionals about their health status and options;
 - Better understand how their status compares with other patients with a similar condition;

- Share their data and help the broader patient community.
- Allow **health care professionals** to:
 - Track the evolution of their patients;
 - Enable a different outcome-based conversation;
 - Better inform and enhance clinical decisions based on the patient perspective.
- Allow **patient organisations** to:
 - Assess the status and dynamics of their patient population;
 - Increase engagement with other healthcare stakeholders in evidence-based advocacy;
 - Further contribute to improving the healthcare system.
- Allow **health authorities** to:
 - Improve the quality of care through better and more transparent evidence of patient measures and outcomes;
 - Drive research agendas and investments in the right areas;
 - Ensure the sustainability of healthcare systems in finding ways to improve the allocation of resources.
- Allow **pharmaceutical companies and other innovative companies** to use data to:
 - Enable ethical utilisation of the Observatory data as legally appropriate.
 - Generate insights that can be used to support the design and direction of the development of new treatments.
 - Generate robust evidence that can be used in submissions to regulators, Health Technology Assessment (HTA) agencies and other decision makers.

It is also expected that the pool of harmonised data that will be generated can be shared with other institutions and consortia (see section “Potential synergies with existing Consortia”). Standardised data across geographies can eventually enable comparison of outcomes among different healthcare systems.

Finally, applicants should indicate how their proposal will impact the competitiveness and industrial leadership of Europe by, for example, engaging suitable SMEs.

Potential synergies with existing Consortia

Applicants should take into consideration, while preparing their short proposal, relevant national, European (both research projects as well as research infrastructure initiatives), and non-European initiatives. Synergies and complementarities should be considered in order to incorporate past achievements, available data and lessons learnt where possible, thus avoiding unnecessary overlap and duplication of efforts and funding.

Collaboration agreements

There is potential in particular for important synergies with the Integrated Digital Health Information Project, launched under IMI2 Call 18 Topic 3. It is possible for the Integrated Digital Health Information project to leverage the Observatory platform in order to reach patients, obtain their consent, and analyse their data, and also to obtain access to relevant EHR data for use within the project. At the same time, the Integrated Digital

Health Information project could become an additional important user case for the Observatories and improve their usefulness. Additionally, the perspectives brought by the Integrated Digital Health Information project can contribute to development of the governance and operational model of the Observatories and help future proof them as a neutral guardian of patients' health data which could then be made available in the future with the appropriate safeguards for applications such as those envisaged under the Integrated Digital Health Information project.

As a consequence, both projects should conclude a collaboration agreement to explore such synergies. The respective options of Article 2, Article 31.6 and Article 41.4 of the IMI2 Model Grant Agreement will be applied.

Other potential synergies

The project funded under this topic will build upon applicable methodologies and principles established in particular (but not limited to):

- By projects from IMI2 Big Data for Better Outcomes (BD4BO) programme such as:
 - [EHDEN](#) – for infrastructure and principles of data standardisation;
 - [BD4BO](#) disease-specific projects – for their principles of establishing the usefulness of PROs and RWE in decision making and establishing the value of interventions;
 - [DO-IT](#) – for its informed consent principles and recommendations amongst others.
- Patient engagement projects such as [EUPATI](#) and [PARADIGM](#).
- OMOP CDM (OHDSI) can provide a common model to encode data as well as important analytical tools.

Industry consortium

The industry consortium will contribute to the “horizontal phase” of the project, consisting of the design of the underlying requirements and the business plan (governance, structure, sustainability) by providing the following expertise:

- Medical knowledge for the disease areas;
- Regulatory expertise;
- Health Outcomes and Real World Evidence expertise;
- Legal expertise;
- Financial and business planning expertise;
- Digital technologies expertise;
- Expertise in public/private partnerships related to clinical research in the health care ecosystem.

Moreover, the industry consortium will contribute to the disease-specific “vertical phase” by providing medical, regulatory experts for the disease areas, digital technologies and health outcomes and real world evidence expertise.

Indicative duration of the action

The indicative duration of this action is 60 months.

Future Project Expansion

Potential applicants must be aware that the Innovative Medicines Initiative 2 (IMI2) Joint Undertaking may, if exceptionally needed, publish at a later stage another call for proposals restricted to the consortium already selected under this topic in order to enhance and progress the results and achievements by extending action duration and funding. The consortium will be entitled to open to other beneficiaries as it sees fit.

In the context of this topic, a restricted Call may be launched as part of a future IMI2 JU Annual Work Plan to expand the work to include additional data sources, therapeutic areas and/or health economic analysis, leveraging the success achieved. This would help to maximise the long term impact of the project and to engender continued future successes in making outcomes and value concepts and their application in healthcare and clinics being more fruitful and efficient.

Applicant consortium

The applicant consortium will be selected on the basis of the submitted short proposals.

The applicant consortium is expected to address all the objectives and to make key contributions to the defined deliverables in synergy with the industry consortium which will join the selected applicant consortium in preparation of the full proposal for stage 2.

This may require mobilising, as appropriate, the following expertise:

- Solid experience in measuring health outcomes, creating appropriate methodologies that allow the capture of patient insights and socialising these methodologies appropriately to gain broad acceptance;
- Access to existing real-world data and technology to query the data, together with experience in creating and operating patient registries that capture patient's input and preferences;
- Demonstrated ability to build strong relationships with the health authorities and patient organisations of at least three different countries where there is desire and willingness to co-create these Observatories together with the industry;
- Strong legal skills including GDPR / data governance aspects but also in broader healthcare law;
- Digital architecture and technical skills to set up and/or adapt and operate the appropriate infrastructure in full compliance with GDPR and cybersecurity requirements;
- Technical capabilities to create the right digital solutions that will allow individual patients to monitor their outcomes in accordance with the agreed standards;
- Expertise in data mining, machine learning, computational biology and modelling expertise and resources;
- Biostatisticians and epidemiologists to combine and analyse the data and publish regularly on the outcomes;
- Medical expertise across the disease areas;
- Communications expertise;
- Strong project management expertise.

Very importantly, the applicant consortium should include among their participants, either as members of the consortium or demonstrated willingness to contribute as experts:

- Patient advocacy groups in the respective disease areas and the respective countries to ensure that the Patient Voice is appropriately heard, captured and interpreted;

- National bodies, such as regulatory agencies and/or HTA agencies and/or health authorities in the respective countries/regions to ensure that the Observatories will become part of the national/regional healthcare ecosystems.

Data Management

In their short proposal, applicants should give due visibility to 'data management'. At stage 2, applicants should include a draft Data Management Plan (DMP) in the full proposal, outlining how research data will be handled and made available during the project and after it is completed.

Dissemination, exploitation and communication activities

In their short proposal, applicants should give due visibility to dissemination, exploitation and communication of the project's results. At stage 2, in their full proposal, applicants should further develop these activities.

Partnership with the industry consortium

In their short proposal, applicants should outline a strategy to create a successful partnership with the industry consortium.

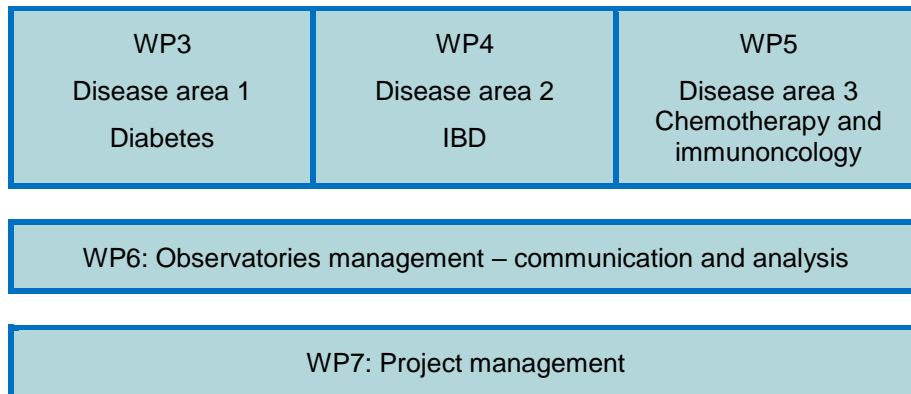
Suggested architecture of the full proposal

The applicant consortium should submit a short proposal which includes their suggestions for creating a full proposal architecture, taking into consideration the industry participation including their contributions and expertise provided below.

In the spirit of the partnership, and to reflect how IMI2 JU call topics are built on identified scientific priorities agreed together with EFPIA beneficiaries/large industrial beneficiaries, these beneficiaries should significantly contribute to the programme and project leadership as well as project financial management. The final architecture of the full proposal will be defined by the participants in compliance with the IMI2 JU rules and with a view to the achievement of the project objectives. The allocation of a leading role within the consortium will be discussed in the course of the drafting of the full proposal to be submitted at stage 2. To facilitate the formation of the final consortium, until the roles are formally appointed through the consortium agreement, the proposed project leader from among EFPIA beneficiaries/large industrial beneficiaries shall facilitate an efficient negotiation of project content and required agreements. All beneficiaries are encouraged to discuss the project architecture and governance and the weighting of responsibilities and priorities therein.

The architecture outlined below for the full proposal is a suggestion. The architecture of the full proposal should be designed to fulfil the objectives and key deliverables within the scope of this proposal.

WP2: Technology-infrastructure



Work package 1: Governance - Sustainability - Capabilities

- Design of the specific governance principles and structures including legal structures, funding and operating model in the given countries in a way to ensure long-term sustainability. This should include the governance and operating procedures for the creation and maintenance of the observatories, including their relationship with patient organisations, health authorities both at regional/national and above country level and commercial entities. Important elements for the design of the appropriate governance model would be:
 - The long-term sustainability of the model;
 - The possibility to scale it to further disease areas;
 - The interoperability of the collected data with health data derived from EHRs, registries, academic researchers etc;
 - The development of a robust consenting process in compliance with GDPR and other relevant legal and regulatory requirements;
 - The creation of an Ethics Council to watch over the Observatories to create strong trust with patients and society.
- Methodology for identifying the appropriate measurement standards ensuring they reflect patients' priorities and validating them. In order for any measurement/reporting tool to be truly useful to patients, it should offer them the possibility to improve their communication with their HCP and/or the health care system more broadly. It is thus an important part of the mission of the Observatories to choose standards that reflect patients' priorities but also socialise these standards with the broader stakeholders in order to gain broader acceptability;
- Identify the capabilities and capacity required for the collection, analysis and dissemination of health data in the observatories, including the required capabilities for data analysis and administration and staff the Observatories appropriately.

Work package 2 – Technology - Infrastructure

Identify the appropriate technology that will allow capturing relevant information from patients and enable real-time information sharing with patients. Set up or adapt the appropriate technology, including tools and platform, that would allow the collection and management of the patient-generated data taking into consideration the possible scalability of the project as well as interoperability of this data with health data derived from other sources (EHR, registries etc.).

Work packages 3 – 5

These work packages will focus on each disease area, aiming to enhance the value of treatment and care for patients through the collection of patient-generated data, the analysis of best care practices as well as the development and validation of digital e-health tools and technologies. The ultimate aim is to increase the wellbeing of patients through improvements in patient care that have been developed with greater insights from patients generated by the Observatories.

Specific common objectives are:

- Identify the appropriate measurement standard for the respective disease/outcome and ensure validation by the stakeholder community;
- Create the methodology to answer the specific research questions identified by the consortium as the most pertinent to the respective disease;
- Provide input to the design of technologies in WP2.

Work package 3 – Diabetes Types 1 and 2

- To focus on the analysis and validation of key outcomes measures and assess their usefulness for diabetes care and contribution to health economic aspects of the healthcare system;
- To use state of the art analytical techniques to demonstrate ideal levels of care based on the validated outcomes data together with other data types such as EHR and patient-generated data;
- To stratify people with diabetes according to outcomes to improve the understanding of diabetic endotypes;
- To develop a digital decision-making system which can be used by healthcare professionals in clinical practice for more personalised treatment of people with type 1 and type 2 diabetes.

The following sub-work packages are proposed to achieve WP3's goals:

- WP3.1: Collecting, refining and validating existing outcome measures to enable solid assessment of the value of a treatment:
 - Weighting of outcome measures and understanding their impact on the quality of life and care of patient segments;
 - Weighting of outcome measures and understanding their appropriateness for the cost of care analyses;
 - Development of a digital decision-making tool, based on outcomes that could be used by HTA bodies to aid in the assessment of new therapies and treatments.
- WP3.2: Analysing and validating clinical, patient-reported and real-world data to enable the development of a novel segmentation of patients to attribute them the right level of care:
 - Deployment of computational biology approaches for assessment and analysis of large multivariate datasets (e.g. outcomes data derived from both EHR and clinical trials) to divide patients into more precise and personalised segments;
 - Development and validation of new recommendation of treatment, care and approaches for the newly defined patient segments based on the comparative assessment of the performance of established treatments for type 1 and type 2 diabetes.

- WP3.3: Development of clinical digital decision-making tool, based on outcomes, for health care providers to aid in the assessment of treatment choice.

Work Package 4 – Inflammatory Bowel Disease

- To establish and validate a key set of key outcomes measures that matter to patients in IBD, especially related to the assessment of disease severity based on patient-reported outcomes;
- To develop digital tools to collect these data directly from patients;
- To assess the acceptance and usability of these tools in patients suffering from IBD;
- To collect a set of patient-generated data using these tools and assess how these outcomes data sets compare to and complement other measures of patient outcome derived from clinical assessments, registries and EHR data;
- To better understand patient endotypes in IBD;
- To better understand how outcomes vary with patient endotypes and clinical practice and assess their potential use for improving patient care and system efficiency in the care of IBD;
- To utilise the PRO data to develop a simple scoring algorithm to indicate a patient's risk of not showing adequate response to their existing IBD therapy (and which could trigger his/her treating physician to re-evaluate treatment strategy);
- To support the development of digital decision-making tools which can be used by healthcare professionals in clinical practice for more personalised treatment based on patient and disease characteristics, treatment history and risk factors.

Work Package 5 – Side effects of chemotherapy and Immuno-Oncology

- To establish and validate a key set of core, patient-relevant, outcomes measures that matter to patients with chemotherapy and immune-oncology side effects, and to develop digital tools to collect these data directly from patients;
- To assess the acceptance and usability of these tools in patients suffering from the side effects of chemotherapy or immune-oncology;
- To collect a set of patient-generated data using these tools and assess how these outcomes data sets compare to and complement other measures of patient outcome derived from clinical assessments, registries and EHR data;
- To better understand how outcomes vary across patients and across clinical practice and assess potential use for improving patient care and system efficiency in the care of cancer patients;
- To better understand patient segments across chemotherapy or immune-oncology side effects;
- To support the development of digital decision-making tools which can be used by healthcare professionals in clinical practice for more personalised treatment of patients with side effects of chemotherapy or immune-oncology.

Work Package 6 – Observatories management: communication and analysis

- Establish the operation of the Observatories including continuous support to patients and other stakeholders for using the technology, collecting feedback and data;

- Generate regular publications to demonstrate the value added of the observatories and the lessons learned;
- Manage the gateway for users of the data (anonymised patient-level data or aggregated data) to be able to access the data;
- Define the appropriate operational and maintenance plan to ensure the technical, organisational and financial sustainability after completion of the project. Explore with partners possible expansion into additional diseases as well as possible integration with EHR and registry data.

Work Package 7 – Project management

Take responsibility for overall project management of the project, including (but not limited to) finance management; meetings management and organisation; administration of communication activities; and supporting the reporting to and communication with the IMI office.

Indicative text