IMPROVE

Framework to IMPROVE the Integration of Patient Generated Health Data to Facilitate Value Based Healthcare

D2.2: Practices report and updates V1

Version 1.0

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** This deliverable was prepared by **PBY** instead of **UU**, as stated in the DoA, since PBY is leading Task T2.4 (Existing practices identification, monitoring and assessment), which is closely aligned with the content and objectives of this deliverable. This change is also reflected in the amendment request with reference **AMD-101132847-5**.*

History of Changes

Date	Version/Page	Change
14.10.2024	0.1	ToC of the deliverable
24.10.2024	0.2	Set-up of the methodology to analyse the practices
14.11.2024	1.0	Final deliverable for peer review
03.12.2024	1.0	Final deliverable for submission

Statement of Originality

This deliverable contains original unpublished work except where clearly indicated otherwise. Acknowledgement of previously published material and of the work of others has been made through appropriate citation, quotation or both.

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Abbreviations and Acronyms

BEAMER	Behavioral and adherence model for improving quality, health outcomes and cost-effectiveness of healthcare
COPD	Chronic Obstructive Pulmonary Disease
EC	European Commission
KPI	Key Performance Indicator
IMPROVE	Framework to IMPROVE the integration of patient generated health data to facilitate value based healthcare
PARADIGM	Patient Preferences in benefit risk assessments during the drug life cycle
PGHD	Patient Generated Health Data
PPI	Patient Preference Information
PREMs	Patient-Reported Experience Measures
PREFER	Patient Preferences in benefit risk assessments during the drug life cycle
PROMs	Patient-Reported Outcome Measures
SISAQOL	Setting International Standards in Analysing Patient-Reported Outcomes and Quality of Life Endpoints
VBHC	Value-Based Health Care
WP	Work Package

Table of Content

Executive Summary

In this report we will provide the main outcomes of Task 2.4 Existing practices identification, monitoring and assessment. Within this task we will identify and analyse important practices across countries and regions, in order to develop a knowledge base of the existing practices that are conducted to develop methods or frameworks for collecting and using patient-generated health data. Data gathering will be done in existing repositories of good practices in different fields and with direct contacts with a wide range of leading regional and national ecosystems. Together with the project manager of these programs we will monitor the practices for a set of predefined and agreed indicators, analyse and assess the effects and efficiencies of the practices implemented that we have identified. Several European (e.g., H2020s, IHI, Horizon Europe's) and national projects have already been identified and this task will continue this exercise over the next 4 years of the project duration. In this first deliverable we will provide a first version of the methodology to be used together with a detailed initial analysis of 5 existing projects that are closely related to the objectives and aims of the IMPROVE project. In future versions of this deliverable, we will refine the methodology and analysis as well as extend it to more projects. Additionally, we will reach out to project coordinators and managers to gather a more in-depth understanding of their work and outcomes as well as to seek active collaborations between IMPROVE and other projects.

Keywords: Scientific; Policy; Practices; Tracker; Artificial Intelligence; Machine Learning

1. Introduction

1.1. IMPROVE approach

The IMPROVE project is dedicated to harnessing the potential of Patient-Generated Health Data (PGHD) through the use of m-health and e-health technologies. This project aims to bridge the current gaps in data utility and fragmentation by integrating and enhancing insights into the daily lives and challenges of patients across all ages who suffer from complex, chronic diseases and comorbidities. The scientific, policy, and practice trackers will be integrated into the platform to ensure a comprehensive analysis of existing activities and work done. By doing so, IMPROVE seeks to extend the capabilities of existing platforms and approaches to Patient-Centered Outcome Measures, enriching them with real-world data that reflect true patient experiences and preferences.

At the core of IMPROVE is the development of a robust platform designed to enable the intelligent use of patient input and generated evidence. This platform will facilitate three key advancements:

- **Enhancing treatment selection**: By advancing the role of patient preferences and experiences in choosing treatments, thereby personalizing healthcare to meet individual needs more effectively.
- **Medical device design improvement**: By incorporating patient feedback directly into the design process, ensuring that new medical devices are more aligned with user expectations and experiences.
- **Accelerating market entry**: By speeding up the introduction of patient-centric and cost-effective advanced integrated care solutions, thus enhancing the accessibility of innovative treatments.

The project will demonstrate the improved clinical adoption of Value-Based Health Care (VBHC) and a higher return on research and innovation investments across various European care settings. With 11 use cases spanning at least five different disease areas, including ophthalmology, oncology, cardiovascular disease, chronic inflammation, and neurology, IMPROVE will employ a diverse range of implementation strategies. These strategies are founded on a design thinking approach, which is essential for testing this innovative framework of data collection and its translation into actionable insights and controlled change.

A substantial contribution from implementation science is also anticipated, aiming to engage all relevant stakeholders to maximize the impact of the IMPROVE initiative on healthcare provision. The project's vision to integrate in-clinic and out-of-clinic PGHD and experiences to harness VBHC will be realized through improved use of Patient-Reported Outcome Measures (PROMs), Patient-Reported Experience Measures (PREMs), Patient Preference Information (PPI), and other PGHD sources. This integration will enable accelerated innovation of cost-effective and personalized patient journeys, offering accurate insights into health conditions, treatment options, and foreseeable outcomes, thus facilitating informed decision-making by patients, their families, and healthcare professionals.

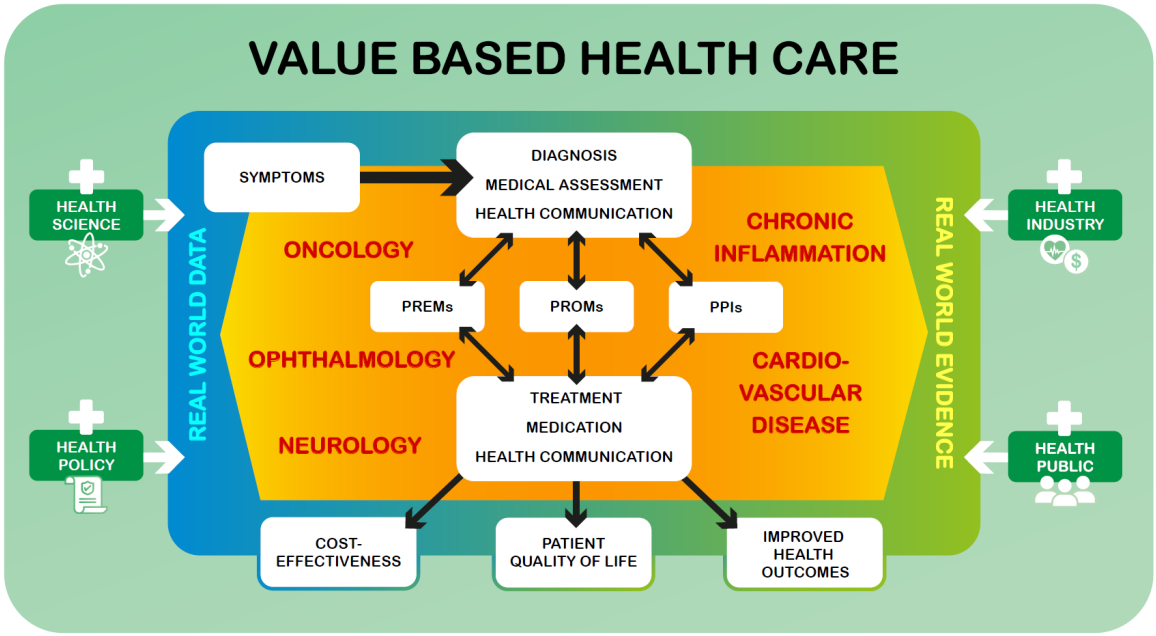


Figure 1 Overview of the project plan and outcomes.

1.2. Overview of the deliverable

It is essential that the project connects also to other initiatives and projects to ensure effective synergies and lessons learned, to make sure that the outcomes are useful and integrated into existing knowledge and processes. In this deliverable, we have established the first version of this work, that will be continued over the full trajectory of the project, updating the current version with more information to support the work in the project. Specifically, the deliverable comprises:

- Section 2: Describing the first version of the methodology used for analysing the projects. In particular, an analysis of practice template is proposed for collecting information from the relevant projects.
- Section 3: Five key projects (also mentioned in the call text) will be analysed following the analysis of practice template.
- Section 4: Concludes the deliverable and defines the next steps.

The ultimate goal of this task is that the information, collected about the practices, is visualized within the practice tracker in the IMPROVE platform.

2. Methodology

In this chapter, we will explain the main methodology to analyse the practices that are considered to be relevant for IMPROVE. In order to standardize the analyses of the practices, we provide here an Analysis of Practice Template:

Analysis of Practice Template

1. Project Overview

- **Title**: The formal title of the research project.
- **Principal Investigator(s)**: Name(s) of the lead researcher(s).
- **Consortium partner(s)**: Organization(s) or institution(s) involved.
- **Funding Source(s)**: Identify funding agencies or sponsors.
- **Project Duration**: Start and end dates of the project.

2. Methodology

- **Summary of the project**: Short summary of the project
  - **Research Problem**: Clearly state the central problem or issue being addressed.
  - **Objectives**: Specific goals of the project.
- **Population, Disease Area and Sample**: Population, sample size, disease area(s).
- **PGHD used**: What kind of PGHD is mainly analysed in the project.
- **Data Collection Methods**: Tools and techniques for data collection (e.g., surveys, interviews, experiments, archival research, etc.).

3. Results & Findings

- **Key Findings**: A summary of the main findings (if available).
- **Data Representation**: Any charts, graphs, or tables that represent the data (if available).
- **Patterns/Trends**: Noteworthy patterns or trends observed from the data.

4. Discussion & Conclusion

- **Interpretation of Findings**: Discuss the meaning and implications of the results in relation to the IMPROVE project
- **Gap analyses and Implications for Future Research related to IMPROVE**: Discuss any limitations or constraints exhibited by the project as well as recommendations for future studies or areas for further investigation.

The template is divided into four main categories. The first category aims at collecting high-level descriptive data about the project such as the name, funding source and a brief project summary. Subsequently, the second category concerns data regarding the methodology, gathered with focus on the research problem and how it is addressed. To further collect relevant data for IMPROVE, the type of Patient Generated Health Data used in the project, data collection methods as well as population, disease areas of interest and samples for empirical validation are considered. Such data will be

important for categorizing and possibly allowing for the searchability of practices within the practice tracker of the IMPROVE platform. The third part records the results generated by the project up to this point in time. Since many of the projects are ongoing, this section will be updated with each deliverable related to this task. Finally, the final section of the template considers how the project relates to IMPROVE and what the implications are for IMPROVE. Specific emphasis is to identify gaps that IMPROVE can fill and resources that the other projects can bring. In this way, synergies of the projects are identified which will be explored in the future by engaging with the other projects. The projects' and IHI webpages as well as the CORDIS database are used for collecting the relevant data about the projects.

3. Analysis of Practices

In this section we analyse five significant relevant projects that are highly relevant to IMPROVE: PREFER, BEAMER, Gravitate-Health, SISAQOL, and PARADIGM. The projects are all ongoing IMI projects that approach and use Patient Generated Health data in different ways which makes them an excellent starting point for mapping practices and for subsequent engagement relevant to IMPROVE. These projects (PREFER, SISAQOL, PARADIGM) have been selected because they have been mentioned in the call text of this project to be working together to find potential synergies or synergies between these projects are already happening (BEAMER, Gravitate-Health) because several IMPROVE consortium members are also active in these projects.

3.1. Practice PREFER

prefer. PATIENT PREFERENCES

1. Project Overview

- **Title**: PREFER - Patient Preferences in benefit risk assessments during the drug life cycle
- **Website:** https://www.ihi.europa.eu/projects-results/project-factsheets/prefer
- **Principal Investigator(s)**: Mats G. Hansson, Uppsala University, Coordinator - Conny Berlin - Novartis Pharma AG
- **Consortium partner(s)**: Partners - PREFER
- **Funding Source(s)**: IMI/IHI
- **Project Duration**: 1-10-2016 until 31-05-2022

2. Methodology

- **Summary:** Sometimes more than one treatment option is available to patients. A series of factors go into deciding the best treatment path for an individual – for example: efficacy and availability of treatment, access to treatment, risk and severity of side effects, convenience, age, expense, how invasive the treatment is, what the follow-up is like. New therapies should not only target clinically relevant outcomes but also what the patient feels is important. For instance, patient preference researchers learned from patients with chronic obstructive pulmonary disease (COPD) that it is not only clinically relevant outcomes – like lung function and hospitalisations – that are important. Patients also want treatments that decrease excessive coughing and mucus secretion, which disturb their usual daily activities. Where there are a variety of treatment options available, trade-offs come into play. For instance, for some patients with rheumatoid arthritis, physiotherapy and exercises might be the preferred route, whereas others will opt for a pill, while still others could benefit more from injections into the affected joints. For each of these options there are trade-offs – pills are easy to take but come with side effects; injections are fast-acting and potent, but are also invasive, and the patient has to travel to the medical centre regularly for treatments which may be inconvenient. Knowing what preferences patients have can aid drug development. There is little point in a pharmaceutical company spending time, money, and resources on developing a treatment if patients struggle with it because it does not address their needs sufficiently. Integrating patient preference studies into the existing development programmes can help researchers to understand what is most important for patients and the acceptable trade-offs. Three main

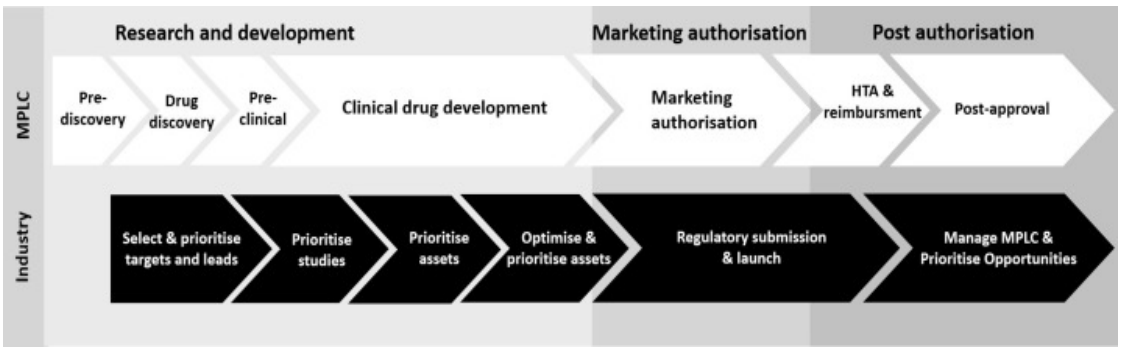
disease areas – lung cancer, rheumatoid arthritis and neuromuscular disorders were the initial focus for patient preference case studies in the PREFER project. However, the project finally compiled a series of ten case studies covering not just these disease areas but also others, such as diabetes, chronic obstructive pulmonary disease (COPD) and haemophilia. The complete set of case studies can be found on PREFER's website. The project needed to assess the variety of methods for conducting patient preference studies that the literature reviews had outlined. Ten qualitative methods and 23 quantitative methods were identified to explore and elicit patient preferences. Some of the methods were then evaluated in case studies.

  - **Research Problem**: To better understand Patient Preferences in benefit risk assessments during the drug life cycle.
  - **Objectives**: To establish better understanding of Patient Preferences in healthcare provision, mostly medication related. The end outcome were recommendations to be used for several stakeholders.

- **Population and Sample**: Patients in lung cancer, rheumatoid arthritis, neuromuscular disorders, diabetes, chronic obstructive pulmonary disease (COPD) and haemophilia.
- **PGHD used**: Mostly PPIs are used.
- **Data Collection Methods**: Literature reviews, surveys, interviews, and stakeholder sessions.

3. Results & Findings

- **Key Findings**: The recommendations developed by PREFER and the Qualification Opinion from EMA are the ultimate outputs of the project. Based on stakeholder needs, preference study methods and case studies, these recommendations serve not only to aid decision-makers to decide when and how to elicit and integrate patient preferences into medicine development, but also to outline under which circumstances patient preference studies are necessary, and what type of methods to select. There are circumstances where a patient preference study is not necessary, and these are also laid out in the recommendations. For instance, new patient preference studies are not likely to add value if one treatment option is clearly preferable to another; if there are no side effects or disadvantages with a new treatment; or where patient preference is clear from previous high-quality and up-to-date research. In circumstances where the patient has no choice – for example, a surgeon selecting which tool to use – it is also not helpful to run a patient preference study. PREFER identified 15 critical points in the medical product life-cycle where patient preference studies should be considered.
- **Data Representation**: See below.



Whichello, C., Bywall, K. S., Mauer, J., Stephen, W., Cleemput, I., Pinto, C. A., ... & Veldwijk, J. (2020). An overview of critical decision-points in the medical product lifecycle: Where to include patient preference information in the decision-making process?. *Health Policy*, *124*(12), 1325-1332.

- **Patterns/Trends**: Working together with stakeholders PPIs have been used to support decision-makers and patients in medicine development.

4. Discussion & Conclusion

- **Interpretation of Findings**: The consortium has found a way how to integrate PPIs in the full medical product lifecycle and published several important scientific publications, webinars, and recommendations about this.
- **Gap analyses related to IMPROVE**: The consortium only focused on PPIs and focused on only medication treatment. IMPROVE will focus on the full procedure on all patient-generated health data, including other forms of treatment (e.g., lifestyle, sleeping).

3.2. Practice BEAMER

1. Project Overview

- **Title**: BEAMER
- **Principal Investigator(s)**: Francisco Lupiáñez-Villanueva, PredictBy. Coordinator – Giuseppe Fico Universidad politécnica de Madrid.
- **Consortium partner(s)**: Who's Behind BEAMER - Project Partners and Team
- **Funding Source(s)**: IMI/IHI
- **Project Duration**: 01/09/2021 to 31/08/2026
- **Summary of the project**: Around half of all patients do not take their treatment as prescribed. This 'non-adherence' to treatment can have a dramatic impact on patients' health and quality of life, resulting in avoidable hospitalisations and contributing to an estimated 200,000 deaths annually in the EU.

  Currently, we do not fully understand all the factors that influence patients' decisions regarding their treatments. The BEAMER project aims to add to our understanding of the factors that influence patient adherence across disease areas and deliver guidance that various stakeholders could use to address patients' needs and boost adherence.

  The project will create a model of the main factors affecting patient adherence to treatment and test it in pilot studies involving 18,000 patients in 6 countries. This will allow the project team to define non-adherence and develop guidance that healthcare stakeholders could transform into tools and solutions to improve adherence. The model will not be disease-specific, but it will be possible for users to add disease-specific elements. This will make the model more widely applicable to different groups of patients.

2. Methodology

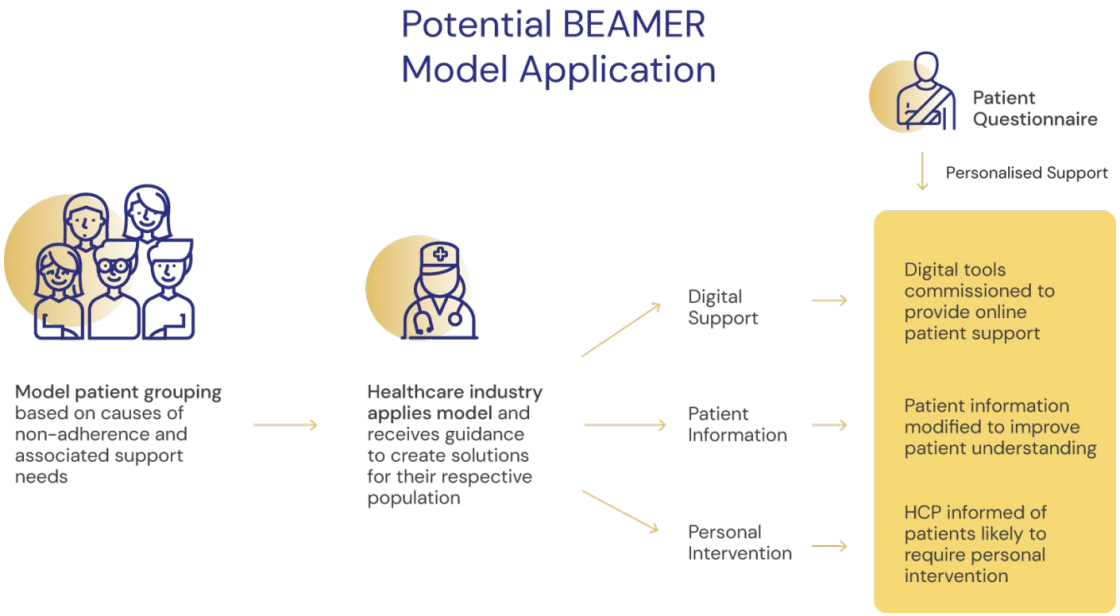
- **Research Problem**:
  1. Around half of all patients do not take their treatment as prescribed. This 'non-adherence' to treatment can have a dramatic impact on patients' health and quality of life, resulting in avoidable hospitalisations and contributing to an estimated 200,000 deaths annually in the EU.
  2. Currently, we do not fully understand all the factors that influence patients' decisions regarding their treatments. The BEAMER project aims to add to our understanding of the factors that influence patient adherence across disease areas and deliver guidance that various stakeholders could use to address patients' needs and boost adherence.
- **Objectives**: The project will create a model of the main factors affecting patient adherence to treatment and test it in pilot studies involving 18 000 patients in 6 countries. This will allow the project team to define non-adherence and develop guidance that healthcare stakeholders could transform into tools and solutions to improve adherence. The model will not be disease specific, but it will be possible for users to add disease-specific elements. This will make the model more widely applicable to different groups of patients.
- **Population and Sample**: Pilot studies conducted with patients with chronic disease mainly from the areas of cardiovascular disease, oncology, endocrinology, immunology, rare

disease, and neurology. Patients are predominantly from Spain, Portugal, the Netherlands, Norway, Italy, or Germany.

- **PGHD used:**
  - PROMs:
    - Adherence measures: TAPQ, MARS-5, Morisky Medication Adherence Scale.
    - Quality of life: EQ5D
    - Psychological and behavioural factors of condition, treatment and health.
  - PREMs:
    - Questionnaires and interviews to evaluate the usefulness of the developed model.
- **Data Collection Methods:** Surveys, interviews, clinical trials, RWD studies, longitudinal studies, literature reviews, stakeholder sessions.

3. Results & Findings

- **Key Findings**: The BEAMER questionnaire has been created and validated for collecting PROMs to explain adherence behaviour. Additionally, the B-COMPASS has been developed that offers a process for eliciting patients' needs that in turn enable support to improve patient's adherence to treatment. The B-COMPASS is disease-agnostic and will cope with the challenges related to the heterogeneity of different contexts and conditions. It offers an elicitation process that is composed of four components that segments the population and predicts relative adherence to treatment of the patients based on the information collected in the BEAMER questionnaire. Additionally, data collection has been standardized by mapping the BEAMER questionnaire to the OMOP Common Data Model.
- **Data Representation**:



- **Patterns/Trends**: Psychological and behavioural factors explain adherence behaviour and the B-COMPASS enables target patient support.

4. Discussion & Conclusion

- **Interpretation of Findings**: PROMs related to adherence to treatment are collected and persisted in a standardized way in the structure of the OMOP Common Data model. Additionally, EQ5D is collected. The interrelation of different PROMs is studied, which is of importance for IMPROVE.
- **Gap analyses and future directions for IMPROVE**: Interrelationships of specific PROMs, PREMs and PPIs is not yet discussed in detail in the IMPROVE project so far, while in BEAMER the standardization of the BEAMER questionnaire was done by mapping the questions to the OMOP CMD and was carried out by the consortium. Potentially, IMPROVE needs to create such vocabularies for different PROM, PREMs, and PPI to adhere to standard data models used. Setting these data standards, if they are absent, would be a very good way for IMPROVE to make impact and be used in practice.

3.3. Practice Gravitate Health

1. Project Overview

- **Title**: Gravitate Health
- **Principal Investigator(s)**: Anne Moen – Universitet I Oslo - coordinator
- **Consortium partner(s)**: https://www.gravitatehealth.eu/partners/
- **Funding Source(s)**: IMI
- **Project Duration**: 01/11/2020 to 31/10/2025

2. Methodology

- **Summary of the project**: Vast amounts of information on medicines are available, especially online, but it is very hard to know what is reliable or even relevant for a specific patient. And, while each medicine comes with a detailed information leaflet, patients often find these difficult to read and understand. This is an issue because when patients lack important information about their treatments, they may not take them correctly and this can result in further health problems. The OECD (Organisation for Economic Cooperation and Development) estimates that poor medication adherence may contribute to around 200 000 premature deaths in Europe every year.
- **Research Problem**: Vast amounts of information on medicines are available, especially online, but it is very hard to know what is reliable or even relevant for a specific patient. And, while each medicine comes with a detailed information leaflet, patients often find these difficult to read and understand.

  This is an issue because when patients lack important information about their treatments, they may not take them correctly and this can result in further health problems. The OECD (Organisation for Economic Cooperation and Development) estimates that poor medication adherence may contribute to around 200 000 premature deaths in Europe every year.

  Objectives: The aim of Gravitate-Health is to develop a digital health information tool called the Gravitate Lens (G-Lens). As the name suggests, the G-Lens will focus (but not conceal or filter) approved information on medicines and guide patients to understandable, trustworthy, up-to-date information that meets the patient's needs and fits with their health context and literacy levels. The functionality of the G-Lens will be supported by an open-source digital platform.

  More broadly, the project hopes to demonstrate that by making information on medicines more accessible and understandable, patients will be more likely to take their medicines correctly, resulting in better health outcomes and quality of life.

  In addition to the open-source platform underlying the G-Lens, the project will produce a white paper with recommendations on realistic strategies on the future use of digital services like electronic product information can be used to further minimise the risks associated with incorrect adherence to advice on medicines.

  - **Population and Sample**: healthy citizens, chronic patients, and informal care from Norway, Sweden, Denmark, Italy, Spain, Portugal, the Netherlands, Ireland, and USA.

- **PGHD used**: PROMs used for the focusing, to characterize/segment patients and QALY as well as medication adherence/compliance as outcome. PREMs for the experience of G-Lens.
- **Data Collection Methods**: Questionnaires, EHR, interviews, stakeholder engagement activities, RWD studies and trials.

3. Results & Findings

- **Key Findings**: The Gravitate-Health project integrates stakeholder requirements, user-centered design, and KPIs into the Federated Open-Source Platform (FOSPS) and G-lens® services for self-care and active treatment. Two minimum viable products (MVP1 and MVP2) were developed to improve ePI information accessibility, including multilingual support. Testing by the User Advisory Group (UAG) and consortium will guide the next version, MVP3. Advanced testing and controlled trials with synthetic and real patient data are planned, alongside selecting test sites for large-scale evaluations. With new partners and global standards adoption, the project enhances ePI access in different languages. Advisory boards ensure ethical compliance, while ongoing outreach through the "Gravitate-Health Triangle" supports sustainability, engagement, and future ePI adoption. The HIMSS community and the newly formed ePI Technology Community help extend project impact.
- **Data Representation**: N/A
- **Patterns/Trends**: Data collection in progress.

4. Discussion & Conclusion

- **Interpretation of Findings**: PGHD is not utilized in the G-lens solution per se, but in the evaluation of the digital solution. Consequently, IMPROVE has the potential of be of great value to the Gravitate health project for collecting PROMs and PREMs related to the evaluation of the G-lens.
- **Gap analyses and implications for future research related to IMPROVE**: Initial steps of defining data requirements for evaluating (digital) health services has started. IMPROVE should investigate whether standardizing collection of PROMs and PREMs related to the evaluation of digital services should be done. Additionally, HTA will be addressed in IMRPOVE via the cost-effectiveness tool. However, more effort could be spent in this direction as well.

3.4. Practice SISAQOL

1. Project Overview

- **Title**: SISAQOL - Setting International Standards in Analysing Patient-Reported Outcomes and Quality of Life Endpoints
- **Principal Investigator(s)**: European Organisation For Research And Treatment Of Cancer Aisbl - coordinator
- **Consortium partner(s)**: SISAQOL-IMI | IHI Innovative Health Initiative
- **Funding Source(s)**: IMI
- **Project Duration**: 01/01/2021 to 31/12/2025

2. Methodology

- **Summary of the project**: In drug development, it is important to find out how treatments affect how patients feel and function in their daily lives. This information is also essential when weighing up the benefits and risks of a medicine. In practice, it is rather difficult to obtain this information and communicate it clearly and simply.

  The aim of SISAQOL-IMI is to develop recommendations on how to analyse and interpret data on health-related quality of life (HRQOL) and patient reported outcomes (PROs) in cancer clinical trials.

  To do this, the project will seek to achieve consensus internationally and across stakeholder groups on the optimal use of PROs in cancer clinical trials, and gain clarity on the research objectives for the use of PROs in trials, including the definition of 'clinically meaningful change'.

  The project also aims to improve the statistical analysis of PROs in cancer clinical trials, and standardise the way findings are reported, presented and visualised. The standards should be endorsed by all relevant stakeholders.

  Furthermore, the project plans to develop educational tools based on the standards; these tools will hopefully help to improve patients' understanding and empower shared decision making.

  Ultimately, the tools and resources developed by SISAQOL-IMI should ensure that cancer clinical trials accurately capture how patients feel or function during treatment. This in turn will aid in decision making for regulators, health technology assessment bodies, and, crucially, improve patient satisfaction. The findings may also be applicable to clinical trials in other disease areas.

- **Research Problem**: In drug development, it is important to find out how treatments affect how patients feel and function in their daily lives. This information is also essential when weighing up the benefits and risks of a medicine. In practice, it is rather difficult to obtain this information and communicate it clearly and simply.

Objectives: The aim of SISAQOL-IMI is to develop recommendations on how to analyse and interpret data on health-related quality of life (HRQOL) and patient reported outcomes (PROs) in cancer clinical trials.

To do this, the project will seek to achieve consensus internationally and across stakeholder groups on the optimal use of PROs in cancer clinical trials, and gain clarity on the research objectives for the use of PROs in trials, including the definition of 'clinically meaningful change'.

The project also aims to improve the statistical analysis of PROs in cancer clinical trials, and standardise the way findings are reported, presented and visualised. The standards should be endorsed by all relevant stakeholders.

Furthermore, the project plans to develop educational tools based on the standards; these tools will hopefully help to improve patients' understanding and empower shared decision making.

- **Population and Sample**: Stakeholder consultation from the Netherlands, Croatia, Spain, USA, Belgium, Austria, Norway, Denmark, Canada, UK, Germany, Switzerland, Japan, Australia, & France. Stakeholders from Academia, non-profit organizations, SMEs, regulators, HTA bodies and patient representatives. Focused on oncology.

- **PGHD used**: PROMs with particular focus on health-related quality of life.
- **Data Collection Methods**: Stakeholder engagement, interviews, online surveys.

3. Results & Findings

- **Key Findings**: The SISAQOL-IMI Consortium generated its final set of recommendation statements in 2023. This final set of recommendation statements were initially voted through an online survey and then re-voted during the Consensus meeting #3 which took place on 23 – 24 May 2023. Out of 79 statements, 77 were accepted by 2/3 majority.

  In the remaining part of the year, the Consortium focused on refining and finalizing the statements. This involved several processes that were run in parallel: receiving feedback from the Independent Scientific Advisory Board, beta-testing of the recommendations through independent validation, obtaining EMA feedback through the qualification advice and the harmonization of recommendations from WP2 (Randomized Controlled Trials) and WP3 (Single-Arm Studies).

  The results of these processes became available at the end of 2023, and the WP leaders have started evaluating and responding to feedback in preparation for Consensus process #4. The revisions and responses proposed by the WP leaders will be voted on and discussed in the next consensus meeting in May 2024. The Consortium has also started working on its key project outputs, such as the web tool (interactive table), which will allow future users to access a subset of recommendations relevant to their trial objective and endpoint at hand. The first drafts of the graphical templates that will guide visualizations of PRO data from cancer clinical trials have also been circulated within the Consortium and are currently being revised to iron out any discrepancies. Following Consensus meeting #4, the Consortium will work on the finalization of these key outputs and work towards the journal publication with the final recommendation statements.

- **Data Representation**: N/A
- **Patterns/Trends**: Recommendations will become available in 2025 as well as their interactive tool.

4. Discussion & Conclusion

- **Interpretation of Findings**: The project aims to provide guidelines for the use analyse and interpret data of PROs based on desk research and stakeholder consultations.
- **Gap analyses and implications for future research related to IMPROVE**: The project is highly relevant to IMPROVE and at its current stage complementary to what IMPROVE aims to achieve. In particular, IMPROVE can benefit from the recommendations generated by SISAQOL for how to visualize and interpret results for PROMS, possibly extending to PREMs and PPIs as well whenever deemed relevant. Additionally, SISAQOL does not offer a software solution where recommendations are implemented in practice. Therefore, IMPROVE can fill the missing link by incorporating some of the recommendations into the platform. If IMPROVE decides to visualize results of PROMs, active collaboration with SISAQOL should be sought.

3.5. Practice PARADIGM

1. Project Overview

- **Title**: PARADIGM
- **Principal Investigator(s)**: European Patients' Forum and EFPIA
- **Consortium partner(s)**: Project partners - PARADIGM
- **Funding Source(s)**: IMI/IHI
- **Project Duration**: 01.03.2018 until 31.08.2020

2. Methodology

- **Summary of the project**: PARADIGM's mission is to provide a unique framework that enables structured, effective, meaningful, ethical, innovative, and sustainable patient engagement (PE) and demonstrates the 'return on the engagement' for all players. The objective is to develop much needed processes and tools for three key decision-making points: **research priority setting, design of clinical trials and early dialogue**. Building on advances at international level, PARADIGM will integrate the needs, perspectives and expectations of all actors (including vulnerable populations) involved and will also produce a set of metrics to measure the impact of patient engagement.
- **Research Problem**: Patient engagement is needed and essential, but fragmented and unclear how to implement and measure. PARADIGM aims to create a framework to make this understanding and implementation more effective.

  Objectives: PARADIGM's main objective is to provide a framework that will enable a meaningful, impactful, ethical and sustainable patient engagement.

  Population and Sample: Stakeholders.
- **PGHD used**: Not clear.
- **Data Collection Methods**: Stakeholder engagement, literature reviews, desk research.

3. Results & Findings

- **Key Findings**: The consortium has collated the evidence and experiences to develop a framework to improve the engagement of patients during healthcare delivery. See here the PE Toolbox - PE Toolbox new - PARADIGM
- **Data Representation**: N/A
- **Patterns/Trends**: The toolbox is the main outcome.

4. Discussion & Conclusion

- **Interpretation of Findings**: The framework and toolbox are the main outcomes.
- **Gap analyses and implications for future research related to IMPROVE**: The project is highly relevant to IMPROVE and at it currents stage complementary to what IMPROVE aims to achieve, especially the framework how to engage with patients during research and medicine development and the toolbox that have been developed. In particular, IMPROVE can benefit

from the toolbox and the framework. Additionally, PARADIGM does not offer a software solution or data collection possibilities where recommendations are implemented in practice and how data should be collected and integrated. Therefore, IMPROVE can fill the missing link by incorporating some of the recommendations into the platform and make use of the toolbox that has been developed.

4. Conclusions

This deliverable summarizes the first steps in establishing the analysis of practices relevant for the IMPROVE project. In particular, a methodology was outlined with the establishment of an Analysis of Practice Template to be used for analysing the existing relevant projects and to be able and extract the relevant practices. Subsequently, five key projects were analysed: PREFER, BEAMER, Gravitate-Health, SISAQOL-IMI, and PARADIGM. There is quite some heterogeneity in how the projects incorporate Patient Generated Health data in their work and their ultimate goals for their usage. Therefore, the insights generated for IMPROVE are rich and provide an initial but broad picture. Specific emphasis can be put on the SISAQOL project that aims to provide recommendations for how to analyse and interpret PROMs data. These insights can be incorporated into the IMPROVE project and platform if deemed desirable.

The current deliverable is the first in a series of deliverables mapping practices relevant to IMPROVE. Subsequently, the proposed methodology will be validated with stakeholders and fine-tuned to meet the specific needs of IMPROVE and the practice tracker. Furthermore, many of the projects analysed are ongoing and therefore updates of the information gathered will be provided. Many additional projects have been identified as potentially relevant to IMPROVE and these projects will also be analysed to create a comprehensive overview of relevant practices to be implemented in the practice tracker. This will also allow for extensive engagement between IMPROVE and other projects.

About IMPROVE

IMPROVE aims to be a dynamic, ready-to-use framework for seamlessly integrating patient-reported information. This adaptable system constantly evolves with the latest evidence, using PGHD and health system data to provide cost-effective solutions for diverse treatment conditions in real settings. The project follows Ontology, Epistemology, and Methodology principles. Ontology defines structures in patient-reported outcomes; Epistemology ensures valid knowledge; Methodology links techniques to outcomes, systematically addressed in its work.

IMPROVE optimizes patient-reported information in real settings, offering a deep understanding of patient behaviors. The project sets up ontology, epistemology, and methodology to minimize the burden on stakeholders cost-effectively. It adopts a scalable, data-driven approach with NLP-driven knowledge extraction. Real World Data is integrated into the Federated Causal Evidence module for comprehensive understanding. Evidence collected enables visualizing attributes affecting patient-reported outcomes through IMPROVE Engagement Factors and Indicators Knowledge Graphs.

IMPROVE's toolkit includes resources for decision-makers, featuring plausible scenarios via the Copenhagen Method. Patient engagement via the MULTI-ACT model ensures sustainable healthcare aligned with patient priorities. This project delivers a modular, open access strategy, providing a trustworthy ecosystem of evidence-based applications. Patient engagement and co-creation scenarios solidify its role in transforming healthcare research and care.

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