

H₂O

HEALTH OUTCOMES
OBSERVATORY

The health outcomes observatory:
**The power of patient-reported
outcomes**



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1. Executive Summary

Realising the power of patient-reported outcomes

Healthcare systems face unprecedented pressures around the globe. Ageing populations, shortages of healthcare professionals and funding restrictions are creating a threefold set of strains on our healthcare systems. If we want these systems to be sustainable, we must focus our healthcare resources on results.

When it comes to health, patient outcomes must be considered the most important Key Performance Indicator (KPI). This may sound obvious but it is not. Tracking patients' outcomes can be complicated. It requires standardisation of the outcomes to be measured and innovative technologies to smoothen the process.

The **Health Outcomes Observatories (H2O)** are focused on creating a common language to allow patients and physicians to share standardised insights on outcomes. This common language enables patients to articulate what matters most to them, improves the communication between patients and physicians and can enable joint decision-making in healthcare.

Better knowledge of patient outcomes and improved communication between patients and healthcare professionals (HCPs) can enhance health outcomes and enable the optimal allocation of healthcare resources to where these can have the greatest impact.

Patient-reported outcomes (PROs) are foundational to this common language. These are PROs that can be monitored by patients without any intervention by healthcare professionals (HCPs). To do this, patients rely on digital technologies and that's why building a trusted digital ecosystem has been a critical part of H2O's work.

At the same time, by leveraging digital technologies and focusing on creating a trusted digital ecosystem, H2O enables pseudonymised, secure and interoperable PRO data to be accessible across countries. Broader and deeper pools of health data can significantly increase the insights drawn from statistical analysis. H2O's standardisation of health data is therefore a powerful tool that can strengthen both medical research and health policy decision-making.

The H2O initiative has already established a number of National Observatories. And the door is open to more! The more countries that join, the deeper the insights that can be gained. In this White Paper, we describe in detail the benefits that the H2O approach can bring and warmly encourage the setting up of more Observatories in other European countries.



"H2O empowers patients and patient representatives, as the collected insights will help us to identify pain points in our healthcare systems and find remedies to them."

Valentina Strammello, Programme Director, European Patients' Forum.

"PROs are essential as part of the accelerating transition to digital healthcare. This makes H2O an invaluable initiative for developing and identifying patient- and disease-relevant PROs that can be implemented into daily clinical practice across Europe."

Annette Thurah, Professor, Aarhus University Hospital, Institute of Clinical Medicine.

"By standardising PROs for the first time, we researchers can access invaluable data that translates across borders and between systems. And, by combining these real-world insights with clinical data, we will have all the variables we require to make truly evidence-based decisions and predictions. This will be an absolute game changer for researchers all over the world."

Tanja Stamm, H2O Co-lead, Head of the Institute for Outcomes Research, Deputy Director of the Center for Medical Data Science at the Medical University of Vienna & Head of the Ludwig Boltzmann Institute for Arthritis and Rehabilitation.



2. Introduction

2.1. Addressing the current challenges facing healthcare systems

Global healthcare systems are in crisis. The cumulative impact of an ageing population, rising healthcare costs and limited resources threaten their long-term sustainability. The current models are increasingly unviable, something that the COVID-19 pandemic ruthlessly exposed. This has highlighted the urgent need for a comprehensive re-evaluation of how we deliver healthcare.

The reality of these under-resourced systems is that the institutions responsible for healthcare delivery have become fixated on ensuring they capture sufficient funding to remain viable and ensure their own financial longevity, rather than the well-being of patients. Too often nowadays, doctors lack the time and resources to thoroughly address all their patients' needs. According to Professor Dipak Kalra, President of The European Institute for Innovation through Health Data, "HCPs must delicately balance between the humanistic aspect, and the business aspect of healthcare provision".

One of the reasons this suboptimal resource allocation is commonplace is that there is a lack of information on whether the end results of care actually provide the desired outcomes. Without such insights, systems will always struggle for efficiency.

If sustainable healthcare systems are to become a reality, then resources need to be allocated to where they can make the greatest difference; this means focusing on better outcomes, rather than efficiency of throughput.¹ And not just any outcomes; it needs to be those that matter most to patients. However, understanding what these are will require a deeper engagement with patients. Furthermore, leveraging these insights will mean finding a way to standardise how patient-reported outcomes (PROs) are collected and measured.²

The value of such an approach is undisputable; indeed, researchers have noted that "patient engagement in collecting and using relevant health outcomes data and information remains an underutilised strategy for incentivising value-based care in clinical practice".³ Yet, despite patients being willing to measure meaningful longer-term health outcomes - such as symptom severity or functional status over time - such information is rarely collected by health systems.

"Unless you start measuring health in a standardised way, and making the data transparent to support healthcare decision making, then you are just fixing the symptoms arising from the performance challenges faced by healthcare systems."

Nicholas Carney, Associate Global Medical Science Director, Roche.



"All across Europe, countries are experiencing an urgent need for more sustainable healthcare systems. The supply of economic resources and manpower does not meet the demand and there is an issue of over utilisation of care that does not add value."

Diana Delnoij, Chief Scientific Officer of Zorginstituut Nederland (Dutch National Healthcare Institute).

"The challenge of medicine over the last centuries has been to increase life expectancy. The current challenge is to reduce morbidity. In order to do that, we have to empirically capture what morbidity is from the patient perspective. Doing so will automatically make medicine more patient-centered."

Prof Dr Matthias Rose, Medical Director Medical Department, Division of Psychosomatic at Charité – Universitätsmedizin Berlin.

"As long as we continue to fail to use health data, patients will not receive the best treatment."

Tanja Stamm, H2O Co-lead, Head of the Institute for Outcomes Research, Deputy Director of the Center for Medical Data Science at the Medical University of Vienna & Head of the Ludwig Boltzmann Institute for Arthritis and Rehabilitation.



1. Porter, M. E., & Lee, T. H. (2013). The strategy that will fix health care. Harvard Business Review, 91(12), 24-24.

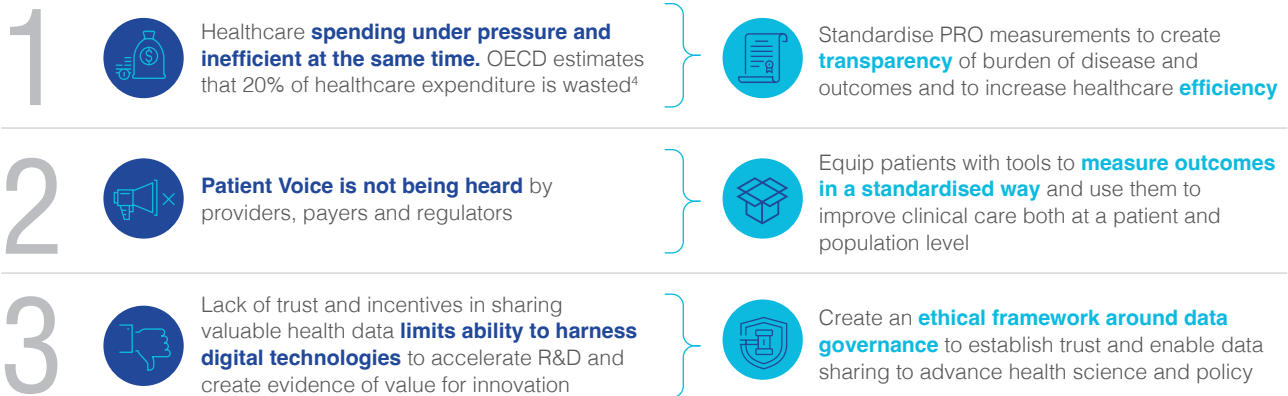
2. Stamm, T., Bott, N., Thwaites, R., Mosor, E., Andrews, M. R., Borgdorff, J., ... & Styliadou, M. (2021). Building a value-based care infrastructure in Europe: The Health Outcomes Observatory. NEJM Catalyst Innovations in Care Delivery, 2(3).

3. Stamm, T. et al. (2021) Building a Value-Based Care Infrastructure in Europe: The Health Outcomes Observatory, in Nejm Catalyst, Innovations in Care Delivery (June 9, 2021)

Figure 1: The challenges facing healthcare systems⁴

The widespread problem





Our ambitious solution



Directing resources to the most-valued outcomes for patients is the best way to deliver effective change, making healthcare systems more efficient and sustainable. The most promising approach for achieving this is through Value-Based Healthcare (VBHC). VBHC can restructure healthcare systems in such a way that business- and human-centred models are not mutually exclusive. By optimising how resources are deployed, VBHC benefits both patients and society.

To address the need for data to power a VBHC approach, key European health stakeholders have launched the Health Outcomes Observatory (H2O) in 2020. H2O is a multi-stakeholder, public-private consortium of patients, patient advocates, clinicians, healthcare providers, researchers and scientists, as well as representatives from the life sciences, pharmaceutical and medical device industries. Its aim is to incorporate data from patient-reported outcomes into healthcare decision making and to put patients at the heart of value-based healthcare. The initiative is led by the pharmaceutical company Takeda and coordinated by the Medical University of Vienna (MedUni Vienna).

H2O is built on the premise that – like water - health data is an essential resource; hence the name for the initiative. Initially funded for five years by the European Innovative Medicines Initiative (IMI) 2 Joint Undertaking, H2O has been constructed in such a way that it both achieves its goal of bringing Patient-Reported Outcomes (PROs) into decision-making and remains sustainable in the future. By giving patients the tools to measure their outcomes in a standardised way, the H2O partners believe that it will allow HCPs and patients to develop a common PRO ‘language’, opening a new world of possibilities, including:

-  improving access to data to inform clinical decisions
-  generating Real World Evidence (RWE) showing the status and dynamics of patient populations
-  providing higher-quality and more-sustainable care through better insights into outcomes
-  supporting the design of, and direction for, developing new treatments.



“Without evidence-based decision making, healthcare systems are unable to meet all the challenges they currently face. Thanks to H2O we can know more about how patients are doing in their day-to-day life. This infrastructure should be used to conduct high-quality studies to generate evidence that is key for good decision making.”

Dr Beate Wieseler, Head of Dept. Drug Assessment at German health technology assessment agency Institute for Quality and Efficiency in Health Care.

“With increased access to data for all stakeholders, H2O is able to shift the power and include all stakeholders in decisions affecting healthcare provision, from patients to payers, from regulators to researchers.”

Herko Coomans, H2O-NL Board of Oversight member, International Digital Health Coordinator at Netherlands Ministry of Health, Welfare and Sport.

“When I first heard about H2O, I thought that this the most intelligent project that I have seen in a long time. H2O gathers patient voices in different countries to collect meaningful health data outside the hospital setting, where it really matters: wherever the patient might be. H2O helps make healthcare systems more patient-centered.”

Prof Dr Matthias Rose, Medical Director Medical Department, Division of Psychosomatic at Charité – Universitätsmedizin Berlin.

4. OECD (2017), Tackling Wasteful Spending on Health, OECD Publishing, Paris, <https://doi.org/10.1787/9789264266414-en>.

There are four initial H2O Observatories, in Spain, Netherlands, Germany and Austria. Each will operate independently as not-for-profit, legal entities specifically created for the purpose of data collection, analysis and evidence sharing to inform clinical practice and healthcare decisions. The model is easily scalable, both in terms of geographical location and the diseases covered.

Uniquely, the H2O observatories will not simply gather any health data, but rather will collect standardised outcome data with a strong emphasis on PROs. Qualitative individual health experiences of the patients' wellbeing - such as the age-old question "How are you?" - will be captured by providing patients the tools to track, monitor and report their health status to their doctors, making these responses measurable and quantitative. Through standardisation, H2O will enable the highest integrity of the data, essentially providing all stakeholders with valuable and interoperable high-quality, real-world data. H2O will also combine these PROs with other data sources, such as ambulatory data streams from mobile devices and wearables as well as clinically captured outcomes from electronic health records.

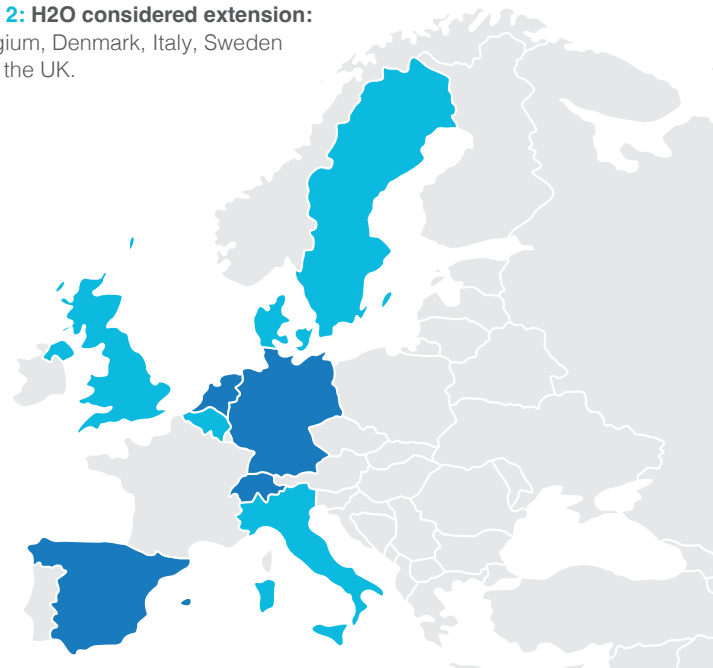
The H2O initiative is focusing initially on diabetes, certain cancers and inflammatory bowel disease. It has evaluated and selected relevant outcome standards that are broadly acceptable to all stakeholders. The first independent national observatories all adhere to national regulatory frameworks and country-specific requirements. A pan-European Observatory, to connect and monitor the activities of the National Observatories, is also being established. This will help harmonise operational principles and core outcome sets to aggregate data at a European level.



"H2O has the potential to provide meaningful data that can be converted to helpful evidence, e.g. if robust studies comparing treatment alternatives in day-to-day life are performed. Using PROs helps us generate evidence that matters to the patients - this is what is needed if we want to provide patient-centred healthcare."

Dr Beate Wieseler, Head of Dept. of Drug Assessment at German health technology assessment agency Institute for Quality and Efficiency in Health Care.

- **Tier 1: H2O first implementation:**
Austria, Germany, Netherlands and Spain.
- **Tier 2: H2O considered extension:**
Belgium, Denmark, Italy, Sweden and the UK.



2.2. The Aim of this White Paper

Given H2O's potential for a profound positive impact, we believe that it can help make sustainable, patient-centred healthcare systems a reality. The aim of this White Paper is to present how H2O will act as a catalyst for this transformation, by using PROs to optimise resource use and allocation and improve the lives of patients.

The White Paper offers a 360° overview of what H2O is, what it does, how it is structured and what it aims to achieve. We have interviewed a number of experts from the four initial H2O national observatories: HCPs, patient representatives, regulators, researchers and industry representatives. The contents are based on their testimonials.

3. Unleashing the power of patient-reported outcomes

3.1. Standardised Patient-Reported Outcomes – the backbone of H2O

PROs are used to assess a patient’s health, quality of life or functional status. These measurements are usually linked to care or treatments a patient has received, for example before and after an intervention. They have the potential to provide an extremely valuable resource, bringing insights from patients about their health and well-being that would otherwise not be captured.⁵

Although PROs have often been collected in the past, there have been substantial difficulties in the practicalities of integrating the data into existing hospital systems, in no small part as consequence of a lack of standardisation. Where the H2O initiative can make a difference by making standardisation a reality by creating a shared language for patients and HCPs. By so doing, the conversations on outcomes become easier and more straightforward to capture, making individual perceptions of health relevant, measurable and usable data.



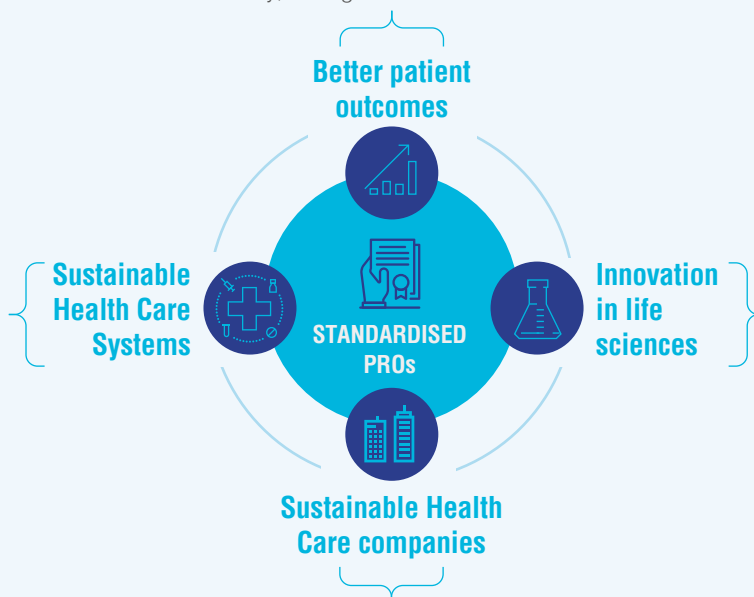
“We are the first public-private consortium - indeed the first group anywhere - to attempt to make it possible for everyone to share a common language around PROs at scale.”

Tanja Stamm, H2O Co-lead, Head of the Institute for Outcomes Research, Deputy Director of the Center for Medical Data Science at the Medical University of Vienna & Head of the Ludwig Boltzmann Institute for Arthritis and Rehabilitation

Figure 2: Standardised PROs

Using structured PROs as a way of communication between HCPs, patients and their carers enables an evidence-based discussion. It creates a common language that amplifies the patient voice in an entirely new way, driving better clinical outcomes

When all parts of the health system have access to standardised PRO data, they see the impact of care across multiple disease areas and populations, giving an unprecedented overview of the real needs of patients and providing an objective way of targeting the pain points in the system



Systematic collection of standardised, structured PROs paired with clinical outcomes can inform design of clinical trials, provide insights into standard of care, reveal patients needs and advance scientific knowledge

Standardised PROs paired with clinical outcomes, can become a new window into the patient experience and outcomes allowing better business decisions to meet unmet patient needs but also allow evidence generation to assess effectiveness of various interventions.

5. PATIENT-REPORTED OUTCOMES: Meaningful insights to improve patients’ lives and patient involvement in health and well-being. Health Outcomes Observatory [Accessed online 15.03.2023]

H2O observatories will achieve this by introducing PROs according to internationally accepted standards (both what to measure and how). Where no such standards exist, the Observatories have introduced and commissioned an objective process for developing them. This was co-created with multiple stakeholders - including health authorities, HCPs, patients and patient groups - to ensure broad acceptance. Furthermore, H2O has worked closely with existing standardisation organisations, such as the International Consortium for Outcome Measurement (ICHOM) and other initiatives, in order to build on their prior work and expertise.



• **Better patient outcomes.** Using standardised PROs among patients, carers and HCPs makes evidence-based discussion simpler. A common language will amplify the value of patients' insights, ultimately improving clinical outcomes for them. PROs allow patients to highlight specific aspects when discussing their health status and reactions to treatments when visiting their HCPs.



• **Innovation in life sciences.** The challenge in research and scientific investigation is to ensure interoperable data and consistent standards for measurements for researchers and scientists. The systematic collection of standardised, structured PROs – when paired with clinical outcomes - can inform the design of clinical trials, by providing insights into standards of care and uncovering patients' needs.



• **Sustainable healthcare companies.** Standardised PROs paired with clinical outcomes can provide fresh insights into how and where existing treatments truly make a difference. This can help healthcare companies, make better-targeted business decisions when developing new therapies, by directing their R&D resources to the areas of greatest unmet need.



• **Sustainable healthcare systems.** When all components of the health system can access standardised PRO data paired with clinical outcomes, the impact of care in multiple disease areas and populations will be visible. This will provide an unparalleled overview of the real



Patient Outcomes can be best measured when one takes into consideration both the Patient Reported Outcomes and the outcomes captured by HCPs, also known as clinically reported outcomes. Within H2O we have emphasized the importance of standardisation in both PROs and clinically captured outcomes. In this White Paper, we place more emphasis on PROs since this is what can be measured by the patients and can create this common language to foster communication and shared decision making.”

Meni Styliadou, H2O Co-Lead, GPL Health Data Partnerships, Data Sciences Institute, Takeda.

“Standardised PROs are similar to lab results; they help us identify issues that may have otherwise been missed, and allow us to screen for things that patients might be too embarrassed to bring up. It helps us monitor the patients while they are home, and may even help us reduce the time that they spend in the hospitals.”

Prof Dr Matthias Rose, Medical Director Medical Department, Division of Psychosomatic at Charité – Universitätsmedizin Berlin.

“H2O collects data directly from patients on those outcomes that matter to them, and it does this in an ongoing, standardised way. Health Technology Assessment (HTA) agencies in Europe don't always have access to relevant data sources or that contain the variables they need. H2O's data is a reliable RWE source that can help HTAs their assessments. Often, HTA agencies give one-off advice, then it goes to a clinical setting. It is important that we follow up what happens and decide whether we should adapt our advice - H2O helps with this.”

Diana Delnoij, Chief Scientific Officer of Zorginstituut Nederland (Dutch National Healthcare Institute).

“H2O standardised PROs allows us, scientists, to make personalized predictions of a patients' future symptoms or quality of life, across Europe.”

Belle de Rooij, Postdoctoral researcher at Integraal Kankercentrum Nederland.

3.2. Patient Empowerment

H2O's ambition is to empower patients to undertake evidence-based monitoring of their own conditions. The unique governance model of the observatories will allow patients to control how their health data is used and give them an influence within H2O.

H2O also allows patients greater control over their treatment, equipping them to discuss their health with their doctor and to advocate for their individual needs. It also gives them control over the use and distribution of the health data collected.

H2O's Patient Agreement

H2O's Patient Agreement provides patients with their first formal involvement with H2O. When the patient signs the agreement, they are provided with control over their own health data. It sets up the permissions to allow the combination of outcome data with electronic health records stored in healthcare facilities. This provides the option of data sharing with accredited institutions for research purposes, with the ability to opt-out where patients would prefer not to be part of research. This is an important expression of the H2O vision; to unleash the power of standardised patient outcomes while keeping patients in control of their own health data. It also offers them a range of options to tailor their use of the H2O-accredited apps.

Knowledge, the start for conversations on eye-level

By using the H2O-accredited apps, patients can track their individual health measurements, identifying patterns that may indicate health episodes or trigger symptoms. Patients can benefit from the H2O-derived insights in several ways. Some patients may wish to track their health outcomes more effectively to provide a basis for a more-detailed discussion with their healthcare providers.

Being able to see changes over time is valuable for many patients. Others may want to compare their diseases and treatments with those in similar situations, to know what works best and use this to discuss a recommended care plan with their HCP.

Furthermore, by integrating their data into the wider H2O system, the patient can compare their health outcomes with those of a wider patient community. This helps them better understand the burden of disease and how their individual experience compares to those with the same condition, providing motivation to improve their treatment.

Patient advocacy and stakeholder engagement

H2O allows for the patient and their representatives to engage more effectively with governments, regulators and relevant institutions. The availability of RWE empowers patient representatives to accurately represent the reality of their day-to-day lives, strengthening their position in evidence-based advocacy for medicines and on reimbursement. They can inform policymakers where further resources within health services are needed, be it additional HCPs, medical devices or therapies to improve quality of life, allowing patients to better manage their conditions and enjoy a higher level of care.



"H2O is about strengthening the active patient, the participating, and engaged patient"

Dipak Kalra, President, The European Institute for Innovation through Health Data.

"In H2O, the patient is in the driver seat. Patient-generated data allows me to provide a higher level of care for the patient"

Linetta Koppert, Oncological surgeon, clinical epidemiologist, MD PhD at Erasmus Medical Center.

"Knowing how other patients are handling the same disease is one of the strongest tools a patient can have. H2O gives this to them, allowing them to take control of their health decisions."

Jolanda Koenders, Head of New Product Planning Central South East Europe, Takeda.

"There is a rebalancing of power towards the patients. They are now the holders and analysers of their own data. It is a true empowerment of the patient."

Herko Coomans, H2O-NL Board of Oversight member, International Digital Health Coordinator at Netherlands Ministry of Health, Welfare and Sport.

"PROs enable us to better represent patients. Governments can directly see the needs of patients, in way that was previously not possible."

Carmen Hurtado, Senior Scientist, JDRF International.

"Access to health data is critical. It better shapes decision, gives voice to patients and allows industry an understanding where we should invest more."

Jolanda Koenders, Head of New Product Planning Central South East Europe, Takeda.

3.3. Governance

Key to patient involvement is trust between the Observatory and the patient. This needs to be sufficient to allow patients to feel safe in allowing their data to be placed under the guardianship of the Observatories. This is why a robust governance model has been developed for both the functioning of the Observatory and for accessing the data collected.

The Observatories' multi-stakeholder approach means that all relevant voices are listened to, helping overcome the hesitancy to share the 'source of power' that is health data.



“With H2O multi-stakeholder governance, we are able to see best practices from around Europe, understand how the data is used in everyday practice, and contribute to the bigger ecosystem.”

Diana Delnoij, Chief Scientific Officer of Zorginstituut Nederland (Dutch National Healthcare Institute).

H2O's governance structure creates a mutually beneficial, 'win-win' scenario for all the stakeholders involved:



Patients

- ✔ **Tracking personal measures** about their condition, such as symptom severity or functional status over time
- ✔ **Having richer and more productive** dialogues with their HCP
- ✔ **Better understanding** the burden of disease, comparing it to others with the same condition and assessing the quality of care they receive



Healthcare Providers

- ✔ **Accessing** structured patient-reported information
- ✔ **Accessing** the patient-reported data between consultations
- ✔ **Having more effective discussions** with patients and enabling better, data-driven decision making and personalisation of patient care
- ✔ **Gaining an overview** of their patients, the patients' views and preferences, and being able to (anonymously) benchmark with similar patients in other practices



Regulators and HTAs

- ✔ **Having access** to high-quality, trustworthy outcomes data, particularly for newly approved medicines and high-cost/high-value treatments
- ✔ **Incorporating patients' PROs and PROMs** in their analyses to highlight areas for potential improvements in healthcare, thus contributing to value based health care



Health Authorities

- ✔ **Supplementing existing data sources** improve understanding of the burden of disease and the performance of the healthcare system
- ✔ **Enabling health authorities** to compare different patient pathways and select the most efficient ones, optimising patient outcomes and costs
- ✔ **Promoting outcomes-based** pricing and reimbursement negotiations, including outcomes-based contracting

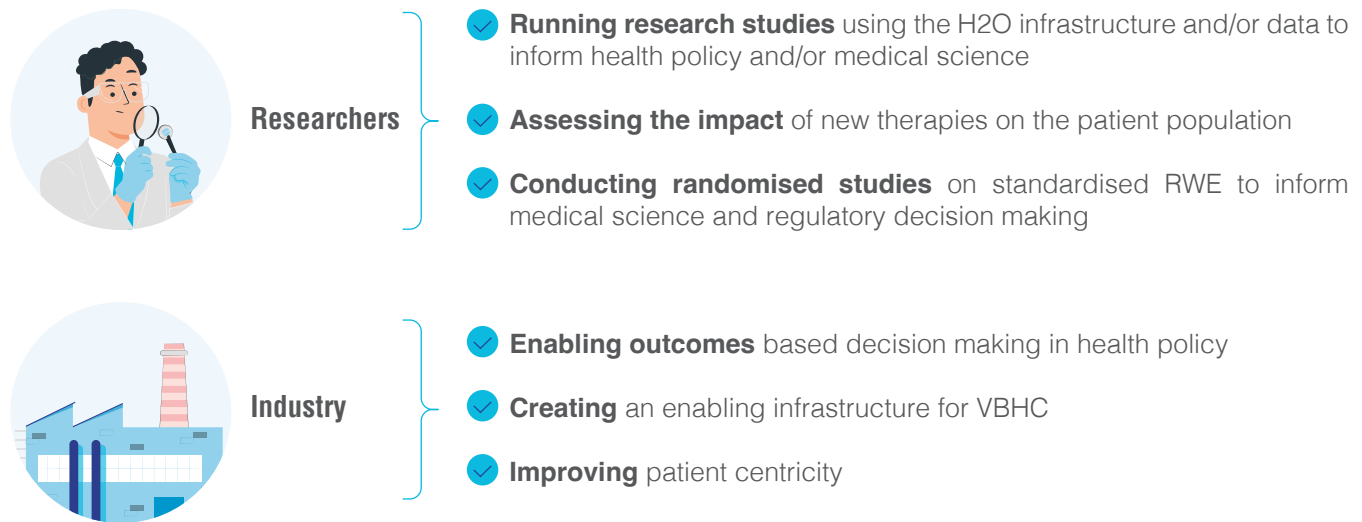
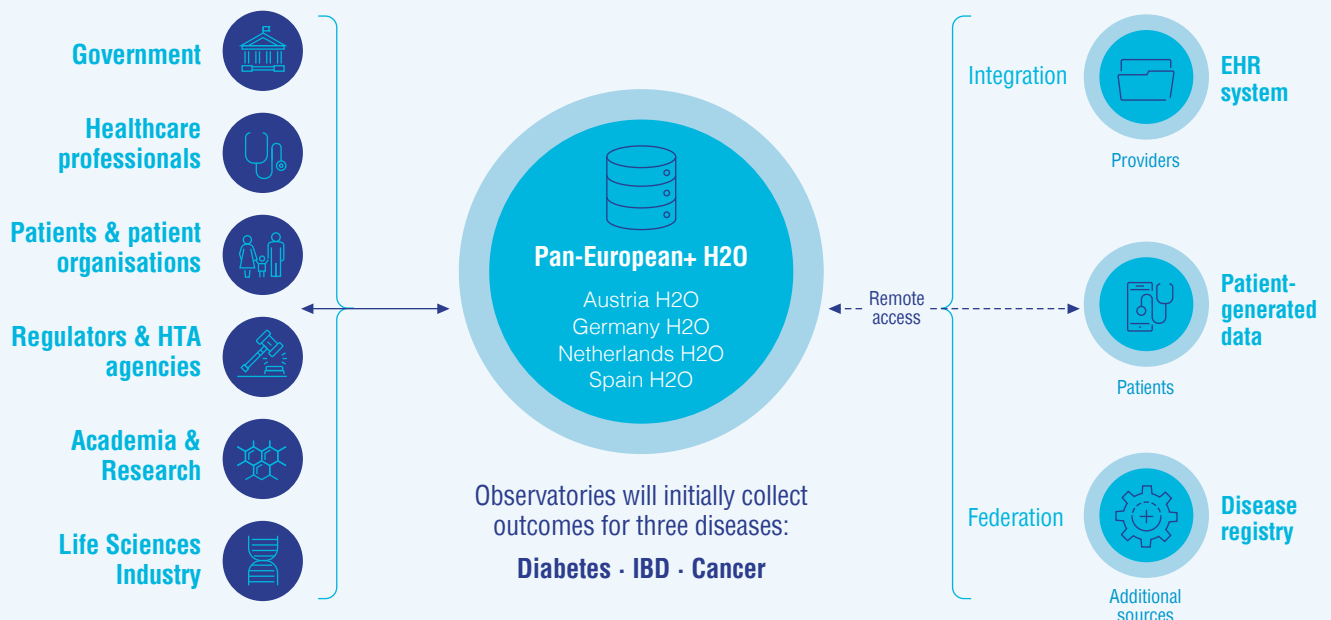


Figure 3: First European scale network for health outcomes data



Observatory Governance – representation and multi-stakeholder approach

The governance board of the Observatories consists of representatives of the five stakeholders, namely patients, HCPs, regulators, society (represented through government) and the life science industry. There are also a managing director and a chief ethics officer who report to the board independently. The ethics officer holds the responsibility for reviewing requests to analyse the data, while the Managing Director ensures sustainability of the Observatory. With both reporting separately to the board, this encourages a balance between remaining viable and continuing to be ethical.

The national observatories come together in the pan-European H2O Observatory. Within this, there are three separate constituencies: the national Observatories, the public sector agencies and the patients. Each constituency appoints representatives to the board, deciding internally who will be the representatives. The voting weight is split as follows; the national Observatories have 60%, the patients have 20%



“H2O allows patients see eye-to-eye with all stakeholders. There is a real sense that H2O works to ensure patients remain the centre of the conversation and are part of it.”

Evelyn Groß, Member of the H2O Patient Advisory Board for Inflammatory Bowel Disease (IBD) and H2O Austria founding board member.

and the public sector 20%. The main objective of this governance model is to strengthen both the patient's voice and the societal voice, as represented through the government. Both patients and society are already represented in the national observatories; providing them with a separate, independent voice in the pan-European Observatory ensures that their perspectives are adequately reflected in all decisions.

The national Observatories authorise the technology partners that operate in their country and ensure that they respect and implement the H2O Principles and standards. The technology partners provide the link to those hospitals and healthcare providers who have joined the H2O ecosystem, managing the data on behalf of patients and in accordance with the H2O Governance Principles. The national observatories act as the guardians of the patient's data and their primary role is to vet the partners that want to join this ecosystem, in order to maintain the necessary level of trust.

It is vital to ensure that the outcomes collected across countries are consistent and coherent, in order to allow for meaningful comparisons. The Pan-European Observatory provides a platform for the national Observatories to agree on the outcomes to be measured and on a consistent approach.

Data Governance – the basis of trust and safety

The multi-stakeholder governance model creates the trust needed to reassure patients that their data are being used ethically and with their full consent. It is designed to give patients full control over the use of their data.

Figure 4: Prioritising each patient's data safety and privacy

Users of H2O can expect that patient data will be stored with the **same level of care** Clinical Records stored within Hospitals.



Data Privacy in Practice

H2O will be following all international and national laws and regulations for keeping information safe, e.g., GDPR

H2O will prevent any attempts to re-identify an individual without explicit consent

Data will be stored on protected computers. We limit and keep track of who can see it and have strict internal policies in place to prevent misuse of data

Experts will regularly monitor systems storing H2O data to make sure they're secure

Data Security in Practice

All names and other information will be removed from data before it is shared with the H2O Observatory, and will only be identified with a unique H2O ID

Patient consent is flexible and dynamic: Patients can determine which of their (pseudonymised) data is shared, and may change their selection at any time

Data will only be shared with approved partners, and access will be tracked and monitored

The governance model is backed by an ethical management system that can only be used with the explicit approval of the patient. H2O collects only the minimum data required from patients necessary to improve care. This ensures that the Observatory is in line with the data protection legislation. Importantly the governance rules are being drafted with input from patient representatives, as part of a continuous dialogue.

Built to last – A sustainable initiative

The success of the H2O Observatories is closely linked to their future viability. While initially funded via the European Commission's IMI, H2O has designed its Observatories in such a way that they will be financially independent once this funding partnership finishes.

The H2O entities as such are not-for-profit; their role is to ensure the overall viability of the ecosystem. H2O has the potential to offer an important source of standardised RWE, including patient-reported outcomes. This can be a valuable source of evidence for several stakeholders. They are currently exploring several models on how to ensure long-term viability, including subscriptions for use of data with different fee structure for different types of stakeholders, charges for ad hoc studies as well as sponsorship by public and private entities.

It is important to remember that hospitals and technology partners need to be appropriately rewarded for their contributions, while patients need to feel comfortable with the overall approach. This is the main role of the H2O entities; to create 'rules of the game' that reassure all participating stakeholders that collaboration and partnership creates greater value than they could achieve individually.

Access to sustainable, transparent funding is key to success of the H2O project; there is thus considerable effort invested in identifying on one hand the right rules for access to data for researchers and on the other hand, appropriate methodologies to reward hospitals and technology providers for their efforts and investments in data collection. The underlying philosophy is to create win-win partnerships that can contribute to the long term sustainability of the overall ecosystem. Multi-stakeholder participation in these reflections is critical. If the rules on data access are too cumbersome, this will jeopardize the attractiveness of the services to researchers. If they are 'too lax', patients and society will be unwilling to support.

The Observatories will also introduce terms and conditions to make it worthwhile for additional partners such as registries, additional healthcare providers and patient organisations to join the H2O ecosystem, helping scale up ethical health outcome analysis.

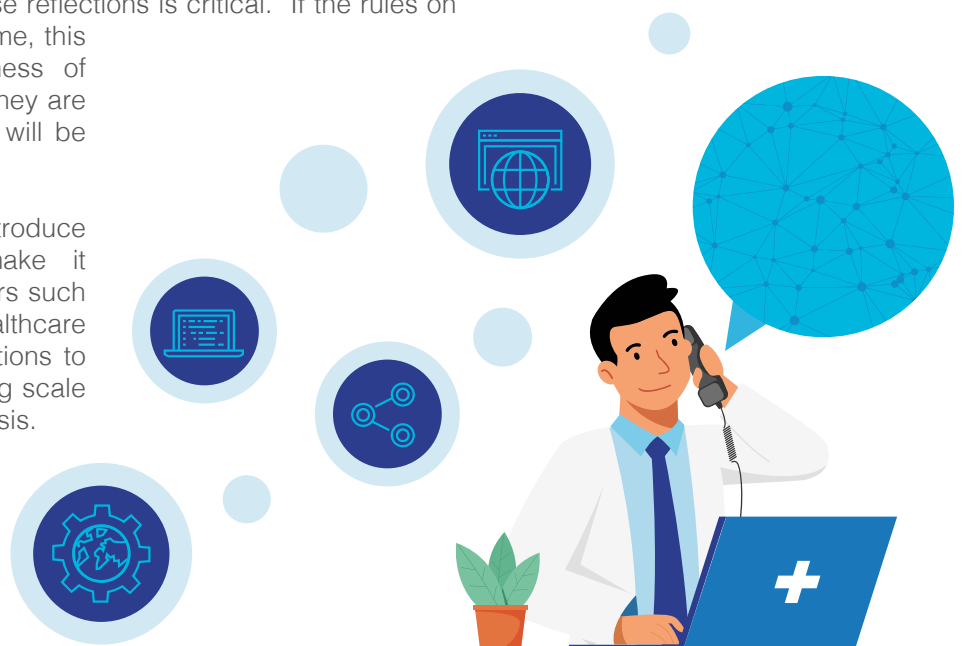


"H2O's multi-stakeholder governance model and public-private partnership offers great potential for scalability and sustainability in a rather rigid and publicly funded system."

Prof Dr Matthias Rose, Medical Director Medical Department, Division of Psychosomatic at Charité – Universitätsmedizin Berlin

"A sustainable governance and funding model is essential to the growth of H2O. Stakeholders can see the benefit brought by the initiative and drive it towards better results for patients."

Meni Styliadou, H2O Co-Lead, GPL Health Data Partnerships, Data Sciences Institute, Takeda



3.4. Building the H2O digital ecosystem

H2O recognised, at an early stage, that its success depended on a technology offer that appealed to both HCPs and patients. H2O has partnered with innovative technology partners with proven track records, which are strongly aligned with H2O values and have broad healthcare system experience in different EU countries.

H2O's back-end data infrastructure providers are the invisible partner, providing safe storage with a high level of data security and integrity for the information shared by patients, their caregivers and HCPs. Essentially, the observatories act as the data trusts that ensure the cybersecurity of the data.

These back-end data enablers are also responsible for managing the health data in line with the permissions granted by the patient. These back-end providers sit between the hospitals and the patient-facing technologies, creating an open-access ecosystem where different innovative solutions can co-exist and compete with each other. The back-end providers also aggregate the data and ensure that it is pseudonymised and made available for other stakeholders.

H2O has worked to ensure that these patient-facing technologies can be trusted, are intuitive and add value. They are the state-of-the-art applications that patients, their caregivers and clinicians use to gather and track their data easily. They are intuitive to use, ensuring the widest possible uptake in the populations. Furthermore, if the patient moves to another country, these solutions ensure that their data can move with them, mitigating the risk of patients losing access to their data.



“Other health data apps exist, but they are not very advanced nor focused on patient-reported outcomes which would have an impact on treatment decisions. Doctors and HCPs were not so interested in using insights from those apps.”

Evelyn Groß, Member of the H2O Patient Advisory Board for Inflammatory Bowel Disease (IBD) and H2O Austria founding board member.





4. H2O's systemic impact

4.1. Improving healthcare systems in Europe and beyond

H2O's decision to embark on this journey of digital health and PROs was not spontaneous; rather, it reflects the fact that the digitalisation of health data is a topic gaining traction globally. Governments are currently investing in infrastructure that facilitates interoperability of health data. Artificial intelligence, machine learning and wearable health technologies are the future – but all of these require well-resourced and functioning digital health data solutions and infrastructure. Governments are taking steps to put this place, but they are seeking to change systems with built-in self-interest. By stressing patient empowerment, H2O aims to leverage the one stakeholder group with most to gain from this transformation.

The European Commission's proposed European Health Data Space (EHDS) is part of the EU's ambition to revolutionise healthcare provision. The EHDS aims to be a "health-specific ecosystem comprised of rules, common standards and practices, infrastructures and a governance framework that aims at empowering individuals through increased digital access to, and control of, their electronic personal health data (primary use of data)... and providing a consistent, trustworthy and efficient set-up for the use of health data for research, innovation, policy-making and regulatory activities (secondary use of data)".⁸

H2O is an excellent demonstration project of what the EHDS could deliver and potentially paves the way for its introduction. The key differentiating factor of H2O is the emphasis on giving patients the ability to measure their outcomes in a standardised way and be part of the digital health ecosystem.

Furthermore, the European Commission is pushing for a new Health Technology Assessment Regulation (HTAR), that aims to "strengthen EU-level cooperation among Member States for assessing new health technologies".⁹ One key element of the HTAR is the Joint Clinical Assessments (JCAs), which systematically assess all available clinical evidence for new medicines. H2O has a role to play in JCA reassessments, by providing longitudinal, standardised, reliable RWE and data. Furthermore, H2O is continuously gathering PROs, thus providing and updating relevant health data throughout the product lifecycle, with unprecedented potential for more-thorough, accurate and transparent assessments.



"The EHDS can learn from the best practices created by H2O."

Dr Beate Wieseler, Head of Dept. Drug Assessment at German health technology assessment agency Institute for Quality and Efficiency in Health Care.

"There is a lot of diversity in healthcare in Europe. H2O can only be successful if standardization efforts are large-scale."

Prof Dr Matthias Rose, Medical Director Medical Department, Division of Psychosomatic at Charité – Universitätsmedizin Berlin



"Pan-European collaboration is key – together we are stronger. Every country has its own challenges on data and data infrastructure. The solution in one country, can be the solution in another; H2O will be an important candidate use case in the EHDS."

Linetta Koppert, Oncological surgeon, clinical epidemiologist, MD PhD at Erasmus Medical Center.

"Any project that offers a vision like that of H2O is relevant for any HTA agency in Europe."

Diana Delnoij, Chief Scientific Officer of Zorginstituut Nederland (Dutch National Healthcare Institute)

8. https://health.ec.europa.eu/ehealth-digital-health-and-care/european-health-data-space_en

9. <https://www.europarl.europa.eu/legislative-train/theme-promoting-our-european-way-of-life/file-health-technology-assessment#:~:text=The%20proposed%20regulation%20on%20HTA,of%20the%20European%20healthcare%20sector.>

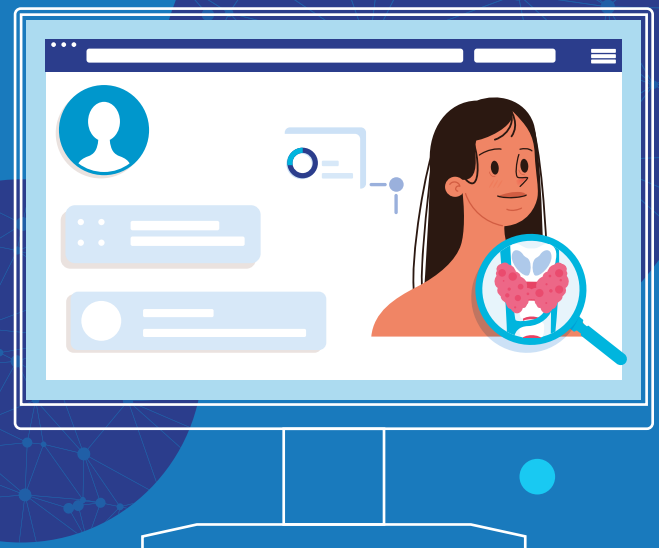
5. Conclusion

H2O is an ambitious, first-of-its-kind initiative. It brings together diverse healthcare stakeholders on national and European levels, putting patients at its core and acting as a beacon for how the future EHDS could look. It uses PROs and data collection to build a system that treats patients as actively involved in how their healthcare is delivered, rather than mere passive recipients.

PROs are the cornerstone of the initiative. However, the other equally important element of this initiative is the Governance Model it introduces. The H2O governance structures in the national and transnational observatories mean that patients can directly influence the direction of the initiative. As they increasingly do in EMA assessments, patients will rightly sit as equals amongst health professionals, researchers, regulators and the life sciences industry. Through the robust data governance, the patient can control who can access their data, how much they want to make available and what it is used for.

The H2O model enables all aspects of VBHC and for all stakeholders. For patients, using their own outcomes and the collective knowledge garnered from those sharing their condition means they can communicate more easily with healthcare providers and seek better treatment. Data sharing allows life-sciences stakeholders to better understand the dynamics of patients and thus develop medicines that work for patients in the ways that they most want. Likewise, researchers can use the data in studies to achieve more concise research outcomes. Doctors will no longer need to determine what has happened since the patients last visit; they can quickly and efficiently read through the available data. All of this works together to the benefit of the patients who need it.

The use of patient-reported outcomes or real-world evidence is growing exponentially. Recognised in the recent European Health Data Space (EHDS) proposal, H2O demonstrates the practical applications of this approach. Cross-border standardisation and transfer of data is revolutionising healthcare, helping it become more data driven, with a growing number of Observatories driving a sustainable approach to healthcare delivery. With the explosive growth in the uptake of technology, the only thing now needed is the right infrastructure to support the approach. This will allow H2O to expand, improving both the treatment of patients and the identification of real-world solutions to pressing health needs and problems.





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