

## Request for Proposal: pan-European Observatory

Please return your proposal to [info@imi-h2o.eu](mailto:info@imi-h2o.eu) by June 14<sup>th</sup>.

### Introduction

The Health Outcomes Observatory (H2O) project will set up and operate national entities – “observatories” – in order to equip patients with digital tools that will allow them to monitor and document their outcomes in a standardised manner. This will enable patients to better manage their health care; it will inform the dialogue between the patient and health care provider; and will allow to better tailor and personalise treatment. A key prerequisite of success for the H2O project is to ensure consistency across the network of national observatories, including:

- Consistency and comparability in the health outcomes measurements and the health outcomes data collected;
- Consistency in terms of approaches to data collection and use; and
- Consistency in code of conduct of observatories and policies for the network as a whole.

To achieve this, the first national observatories will set up an umbrella observatory (the pan-European Observatory) whose main role will be to ensure this consistency in the overall approach. Organisations are invited to respond to this Request for Proposal (RFP) outlining how they would partner with the H2O consortium to establish and operate the pan-European Observatory in line with the strategy and governance principles developed by the H2O project.

### Background

The H2O project has been set up as an IMI (Innovative Medicines Initiative) project running for 5 years from October 2020. Independent, not-for-profit, national observatories are being established initially in four countries – Austria, Germany, The Netherlands and Spain – each focusing on three disease areas: diabetes, inflammatory bowel disease, and cancer. These four national observatories will operate as a cohesive network during the period of IMI funding, and will be sustained beyond this as self-funding bodies.

The mission of H2O is *“to empower patients with tools to monitor their outcomes independently, to promote the use of their outcomes in decision making with clinicians, to create transparency of outcomes to facilitate value based healthcare models and to create an ethical governance model for patient-reported health data in the interest of patients, science and society”*. More detail on the mission and governance principles are given in Annex 1 and Annex 2 respectively.

The H2O project has a number of objectives, including:

- To establish a Health Outcomes Observatory (H2O) as an independent, not-for-profit, legal entity in each participating country, and then to develop and run services in each of the four countries such that these are operational within two years;
- To empower individual patients with the data to better manage their health care; and
- To set up a pan-European observatory that will lead the H2O ecosystem and will extend the H2O concept from its initial focus by adding more disease areas, new data sources, and other settings.

More detailed information on the project, its goals and approach can be found on the [H2O website](#) and the [IMI project page](#).

## The pan-European Observatory

This pan-European Observatory will be established as an independent, not-for-profit organisation with the objective of safeguarding the unique mission of the H2O Observatories to serve patients, society and science.

The pan-European Observatory will play a key role, not only in safeguarding and implementing the H2O mission, but also in leading the direction and operations of the network in the post-IMI funding period. The body will decide the core outcomes measurements, the additional disease areas, the technologies, the code of conduct and the key policies to be implemented in all national observatories that join the broader H2O ecosystem. Additionally the pan-European Observatory will ensure that financial resources are acquired and utilised ethically, effectively and efficiently and that a clear sense of direction and priorities are established and followed by the national observatories.

To achieve this, the pan-European Observatory will be governed by a board representing the three key constituencies of stakeholders: the national observatories, patient organisations, and society at large. For the last constituency, it is expected that a representative of a public sector authority with competence and knowledge of the health care sector, such as a regulator, will bring this perspective. A weighted voting system to reach a balanced representation of all constituencies on the board will be established as follows:

- The votes of national observatories will constitute 60% of all votes;
- The votes of patient organisations will constitute 20% of all votes; and
- The votes of the public authority will constitute 20% of all votes.

A formally articulated agreement (the H2O Constitution Agreement) will set forth the relationships among the organisations, the decision making processes, the policies and procedures that will guide the activities of the H2O network. A first version of characteristics of the Constitution Agreement is given in Annex 3.

It is expected that the pan-European Observatory will be set up as an Association. This appears the most suitable legal form for a number of reasons:

- An Association is a not-for-profit organisation that can use various financial flows, including grants, to be sustainable;
- An Association is an organisation with limited liability, that offers its members protection from personal liability for the activities of the Association;
- The articles of association will reflect the governance model and be compatible with the H2O governance principles;
- New members can be included easily and the Association can expand as needed; and
- In general, formalities are straightforward and registration is simple.

The H2O partners are currently considering the optimal geographic location for this pan-European organisation. Key criteria for the selection are:

- a) The willingness with which a national public sector body with knowledge and competence, including executive authority, in the health sector in the respective country could commit to become a member representing the constituency of public authorities on the board of the pan-European Observatory;
- b) Evidence that the public sector body would demonstrate the willingness and ability to foster a pan-European aligned approach in the activities of the national observatories;

- c) Commitment from the public sector body that it is able and willing to endorse the mission and governance principles of the H2O network and the H2O Constitution Agreement;
- d) Whether local partners in the respective country could provide an office location. The IMI H2O project will be responsible for the direction and initial running of the pan-European observatory; will provide any staffing required; and will manage all communication activities and materials during the period of IMI funding (through to 2025);
- e) Whether, and how, the legal system in the respective country would provide a favourable framework for the operations of the pan-European Observatory; and
- f) A clear commitment to establish a national H2O observatory as part of the H2O network within 3 years of being awarded the pan-European role (for those countries not in the initial four participating in the IMI H2O project).

Interested parties are invited to consider these requirements and to provide a response to this RFP by the 14<sup>th</sup> June. The response should specify: how the pan-European observatory would be established; explain why the country and body would provide an appropriate venue; and detail explicitly how the proposal meets the selection criteria above.

### Process

Please let us know as soon as possible if you intend to respond to this RFP.

In order to respond to specific questions about this RFP, two webinars will be held for all prospective applicants. Where possible, questions should be submitted in advance.

- **Wednesday, 26<sup>th</sup> May, 16.30-17.30 CEST**
- **Monday, 7<sup>th</sup> June, 13.00-14.00 CEST**

Proposals should be submitted to [info@imi-h2o.eu](mailto:info@imi-h2o.eu) by 23:59 CEST on 14<sup>th</sup> June.

The H2O team will review all written proposals. The H2O Steering Committee will make the final decision, aiming to decide on the preferred option in June. Unsuccessful applicants will be notified by email.

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For more details about the H2O project, please visit <https://health-outcomes-observatory.eu/>

### About H2O

H2O is a public-private partnership funded by the Innovative Medicines Initiative and EFPIA members. It was launched on 1 October 2020 and will run for 60 months, through September 2025.

### Supported by a grant from the IMI

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 945345-2. This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA and from Trial Nation and JDRF. <http://www.imi.europa.eu>

### H2O partners

The 23 partners include: Medizinische Universität Wien (**coordinator**) and Takeda Pharmaceuticals International AG (**project leader**); Charité – Universitätsmedizin Berlin, Erasmus Universitair Medisch Centrum Rotterdam, Institut Català de la Salut – Hospital Universitari Vall d’Hebron, Novartis Pharma AG, F. Hoffmann-La Roche Ltd, and Eli Lilly International as **Executive Committee members**; and King’s College London, Katholieke Universiteit Leuven, Forum Europeen des Patients / European Patients’ Forum, The European Institute for Innovation through Health Data, The Hyve BV, TEAM IT Research, Karolinska Universitetssjukhuset, Università Vita-Salute San Raffaele, De Stichting Integraal Kankercentrum Nederland, AbbVie INC, JDRF, Medtronic International Trading SARL, Pfizer Ltd, Sanofi-Aventis Recherche & Development, and Trial Nation as **consortium members**.

## Annex 1 – H2O Mission

The mission of H2O is “to empower patients with tools to monitor their outcomes independently, to promote the use of their outcomes in decision making with clinicians, to create transparency of outcomes to facilitate value based healthcare models and to create an ethical governance model for patient-reported health data in the interest of patients, science and society”. The expanded mission statement includes seven imperatives:

- a) **Empower patients:** engage patients and equip them with digital tools that allow them to monitor their outcomes and to improve their communications with the health care providers (HCPs) and in the long run feel more empowered in the management of their conditions. At the same time, this same data will help to advance science and to create supporting evidence for the development of better health policy.
- b) **Provide full control and ultimately full portability to patients of their outcome data:** patients’ full control of their data is a fundamental principle of this project. To be practical we envision a staged approach whereby patients will have full access to the PROs from the start even if they move hospitals etc. and proceed towards full portability of all their health data ultimately.
- c) **Promote trust through an ethical governance of health data:** Develop an ethical governance model, with input from patients, for the collection and management of health outcome data in order to secure trust with the society and patients while allowing ethical access to this data for the benefit of science, health policy and patients.
- d) **Ensure an ethical framework around access to health outcome data to advance science and health policy while respecting patients’ rights to control their data:** allow bona fide stakeholders with a legitimate interest to have access to the data, subject to an appropriate process and in a sustainable manner, and in compliance with the ethical and legal requirements agreed in the H2O governance model, in order to advance science and improve healthcare management and delivery.
- e) **Create an ecosystem open to all healthcare providers and patients** that creates the right incentives for all stakeholders to ensure interoperability among health data sets and allows the use of technology to analyse outcomes sets by all bona fide researchers;
- f) **Create transparency of outcomes to advance Value Based Health Care:** Encourage transparency of outcomes in order to promote Value Based Health Care and encourage society, HCPs, and all stakeholders to make evidence-based decisions on how to manage healthcare.
- g) **Support evidence-based decisions on health policy:** Publish regularly comparative reports on outcomes to promote best practices and advance science and health policy in the interest of society and patients.

## Annex 2 – H2O Governance principles

While the mission statement states what we are going to do, the principles behind how we do this are outlined in the H2O governance principles. The H2O Consortium has agreed nine governance principles:

### **Principle a: we will set up one Health Outcome Observatory per country.**

Each observatory can support several disease areas. They will be local, not for profit entities with a supervisory multi-stakeholder Board comprising representatives from all key stakeholder constituencies enumerated below. Each constituency will appoint one Board member and all Board members will have equal voting rights. The constituencies represented within the Board are:

- a) Society overall (key representative could be appointed by the government)
- b) Patient Organisations
- c) Medical Professionals
- d) Regulatory Agency and/or HTA authority
- e) Private sector (Life science industry, healthcare consultancies etc.)

The Board will appoint the management structure.

**Principle b: The Health Outcome Observatories will only be truly impactful if there is consistency in the measurements, the possibility to make comparisons, the ability to conduct analysis on large data sets, but also and most importantly, strong trust in the Observatory by society overall and by stakeholders, including patients, citizens, regulators, HCPs, researchers, and industry.**

To ensure this consistency, standardisation and harmonisation, there needs to be a forum and a process where all national Health Outcome Observatories agree on a level of standardisation, on methodologies for measurement of outcomes, on the technologies to ensure data security and integrity but also on the code of conduct for the Observatories towards various stakeholders. Interoperability among the Observatories, consistency in measurement and strong trust in the code of conduct of the Observatories are important prerequisites in order to allow for research and analysis across countries. This will be the role of the pan-European Observatory that will be set up as a separate entity to be the guardian of the Health Outcome Observatory Vision and Mission.

The relationship between the pan-European Observatory and the National Observatories will be governed by a Constitution (Incorporation) Agreement.

**Principle c: the emphasis of H2O is to enable patients to measure patient reported outcomes (PROs). However, the value of the data not only for individual patients and their clinicians but also for health policy and scientific analysis will be significantly enhanced if the H2O tools also incorporate an extract of important clinically captured outcomes.**

To achieve this, the Health Outcome Observatories will focus on creating an ecosystem whereby healthcare providers beyond the founding members are encouraged and incentivised to participate in order to allow their patients to access clinical outcomes through the same tool. An

important focus area for the Observatories will be to work with other partners such as the EH DEN (European Health Data and Evidence Network) consortium, the Data Saves Lives initiative led by the European Patient Forum, public sector entities or additional stakeholders to promote the adoption of a federated approach to data analysis in order to create an interoperable environment and remove technological barriers.

**Principle d: the Health Outcome Observatories will only succeed if there is sustainability in the model while at the same time a robust and ethical governance model for access to data that will build trust with patients and society.**

To ensure sustainability, observatories will need stable income streams. To this end, the Observatories will consider various possible innovative funding models including a subscription and/or sponsorship model to entities interested in the anonymised health data for scientific or health policy research as well as appropriate fee structure for supporting researchers in full research studies. Other novel funding models will also be explored by inviting all stakeholders to look for solutions to the debate about access of health data. There will be different fee structures for different types of stakeholders and consistency in the approach among all Observatories including the pan-European Observatory. The Ethical Council of the pan-European Observatory will assume the overall oversight of the model and will publish appropriate instructions regularly.

The Observatories will also introduce terms and conditions to make it interesting for additional partners including registries, additional healthcare providers, patient organisations etc. to join the H2O ecosystem in order to allow ethical health outcomes analysis on a larger scale.

**Principle e: Health Outcome Observatories need to ensure that patients receive clear value from the Observatory, consent to the data collection and are fully appreciative of the scope and objectives of the Observatories.**

An important priority for the H2O project will be to provide to individual patients the possibility of a dashboard with personalised information on their disease progression, treatment and outcomes in order to allow them to have better discussions with their HCPs. Each individual patient will be able to compare his/her well-being with aggregated data from similar patients with the goal of empowering the patient on his/her journey to better outcomes and better care. With the patient's consent, the relevant healthcare provider will also receive the patient reported information, allowing for a 2-way engagement between patient and healthcare provider on continual improvement of individual care.

To achieve this, the H2O Observatories will have to develop patient consents in line with national laws and regulations and to sustain continuous communication with patients in order to ensure strong patient engagement in the project.

This project will only succeed if patients, as well as other stakeholders, embrace it as a useful approach to improve their communications with the healthcare ecosystem. Patient support is also needed to strengthen the ability of patient advocacy groups to engage in evidence-based advocacy and also contribute to further scientific research in their disease or related health issues.

Thus, it is critical for the Observatories to work closely with the patient community and build a strong relationship of interdependence and trust.

**Principle f: Health Outcomes Observatories will publish regular reports on the status of outcomes in the various diseases in order to promote transparency of outcomes, to support the health authorities in managing healthcare, and to advance science in the disease areas of focus**

One of the key objectives of the Observatory is to encourage transparency of health outcomes in order to allow for better health policy. It is thus important that this becomes an integral part of the project and appropriate resources are being allocated to make sure that reports are being regularly published both with scientific rigour and also in a language understood by the broader public.

**Principle g: Health Outcome Observatories will leverage technologies in order to collect patient reported outcomes and will create an ecosystem that encourages state of the art solutions for patients**

Technology innovation moves fast and it is important to ensure that the best possible solutions reach the patients. To this end, the H2O aims at creating an ecosystem that stimulates innovation and fair competition in possible technological solutions for patients while at the same time ensuring standardization in outcomes measurement and ethical governance of any health outcome data.

**Principle h: The ultimate objective of the H2O is to measure outcomes in all disease areas, including co-morbidities. However, there is a need for prioritisation in order to decide on the roll-out. Decisions on prioritisation will be made within the pan-European Observatory in collaboration with experts and the national Observatories in order to ensure consistency of measurements going forward**

In the short term, the project team will identify the next disease areas for H2O in a pragmatic manner. Moving forward, these decisions will be made through the governing bodies set up by the pan-European Observatory with the participation of all national Observatories. Ensuring the consistency in measurements is a critical prerequisite for success and as a result, these decisions would need to be taken through a rigorous and robust process.

**Principle i: The Observatories will focus on measuring outcomes according to internationally accepted standards of health outcomes (both what to measure and how). Where no standards exist, the Observatories will introduce and commission an objective process for creating such standards**

The H2O intends to collaborate with existing standardisation organisations such as the International Consortium for Outcome Measurement (ICHOM) or other initiatives in order to build on prior work and expertise. Furthermore, the H2O will introduce a methodology and a process to ensure co-creation with multiple stakeholders, including health authorities, HCPs, patients and patient groups, to ensure broad acceptance of outcome measurements.

### Annex 3 – Characteristics of the Constitution Agreement

An pan-European observatory will be established to ensure consistency and comparability in outcome measurements but also in the overall policies, code of conduct and operations of the network of national observatories. This will be a pan-European body initially, but its structure and organisation will be established to allow it to cover all observatories following the H2O mission and governance principle, wherever in the world they are.

Like the national observatories, the pan-European observatory will also be established as an independent, not-for-profit body, and with the scope to conduct revenue raising activities in order to be financially sustainable. The aims of the pan-European organisation are to:

- build a community of patient-centric and outcomes-driven organisations based on transparency and trust to function as a common repository for reports, analytic tools and educational material in connection with national health outcome observatories;
- manage consistency and interoperability across national health outcome observatories and to support the creation of health outcome observatories in other countries in Europe (initially);
- act as a catalyst for future growth of the network of national health outcome observatories; and
- facilitate interoperability, guide reproducibility in other countries, avoid fragmentation using a federated data management approach and promote the benefit of measuring and using outcomes at regional, national, European and global levels.

The organisation of the board of the pan-European observatory will adopt the following representation structure:

- Equal representation of national observatories (60%);
- Representative of patient organisations (20%); and
- Representation of HTA agencies/regulators (20%).

A detailed Constitution Agreement for the pan-European observatory is currently being drafted. This Agreement will not only define the relationship between the national and pan-European observatories, but will also outline a code of conduct covering, amongst other things, the use of and access to data in the network.

Based on a preliminary analysis of the potential legal organisation of the pan-European observatory and the national observatories, we have identified the Association as the most appropriate legal form with the following advantages:

- An Association is a not-for-profit organisation that can use various financial flows, including grants, to be sustainable;
- An Association is an organisation with limited liability, that offers its members protection from personal liability for the activities of the Association;

- The articles of association will reflect the governance model and be compatible with the project principles;
- New members can be included easily and the Association can expand as needed;
- In general, formalities are straightforward and registration is simple;
- The articles of association can reflect our agreed Governance Principles. They can include different ‘blocs’ of stakeholders with separate voting rights (as in Principle a), for example, and allow the multi-stakeholder participation that we would like to achieve in the observatories.

New organisations will be eligible to join the H2O network of national observatories. The pan-European observatory will be responsible for any decision on whether to accept a new member based on the following criteria:

- The organisation is, or will be, an independent, not-for-profit entity;
- The board of the organisation is constituted in line with Principle a) in Annex 2 above;
- The board of the organisation formally adopts the governance principles in Annex 2 above;
- The organisation adheres to the Constitution Agreement agreeing to:
  - safeguard the comparability and consistency of outcome measurements in the interest of patients, society and science;
  - adopt the Code of Conduct that is part of the Constitution Agreement and safeguards how the Observatories handle health data in the interest of patients, society and science; and
  - become a valuable contributor to the governing bodies of the pan-European Observatory and contribute scientific and health policy expertise in order to advance the mission of the Observatory.
- A plan, agreed between the new organisation and the pan-European observatory, is in place. This plan will cover the establishment and growth of the new observatory, including the commitment of at least one major academic hospital to be an active partner in the new observatory;
- The organisation agrees to adopt the technology standards of the H2O network, including for example a common data model (such as the OMOP Common Data Model), the FAIR data principles and interoperability standards such as HL7 FHIR.

Existing members of the H2O network will be required to continue to adhere to the criteria above. Where an existing observatory fails to meet the criteria, the pan-European observatory will be responsible for deciding on remedial measures including, if necessary, expulsion from the network. The process and criteria for sanctions and ultimately, termination, will be outlined in a subsequent H2O Deliverable.