Draft proposal for a European Partnership under Horizon Europe:

European Partnership for Personalised Medicine

EP PerMed

Version 2, January 2022

About this draft

In the course of summer 2021, Commission services asked potential partners to further elaborate proposals for the 2023/24 candidate European Partnerships identified during the strategic planning of Horizon Europe. These proposals have been developed by potential partners based on common guidance and template, considering the initial concepts developed by the Commission and feedback received from Member States during early consultation[1]. The Commission Services have guided revisions during drafting to facilitate alignment with the overall EU political ambition and compliance with the criteria for Partnerships.

This document is a stable draft of the partnership proposal, released for the purpose of ensuring transparency of information on the current status of preparation (incl. on the process for developing the Strategic Research and Innovation Agenda, SRIA). As such, it aims to contribute to further collaboration, synergies and alignment between partnership candidates, as well as more broadly with related R&I stakeholders in the EU, and beyond where relevant. This informal document does not reflect the final views of the Commission, nor pre-empt the formal decision-making (comitology or legislative procedure) on the establishment of European Partnerships.

In the next steps of preparations, the Commission Services will further assess these proposals against the selection criteria for European Partnerships. The final decision on launching a Partnership will depend on progress in their preparation (incl. compliance with selection criteria) and the formal decisions on European Partnerships (linked with the adoption of Strategic Plan, work programmes, and legislative procedures, depending on the form). Key precondition is the existence of an agreed Strategic Research and Innovation Agenda / Roadmap. The launch of a Partnership is also conditional to partners signing up to final, commonly agreed objectives and committing the resources and investments needed from their side to achieve them.

The remaining issues will be addressed in the context of the development of the Strategic Research and Innovation Agenda, and as part of the overall policy (notably in the respective legal frameworks). In particular, it is important that all Partnerships further develop their framework of objectives. All Partnerships need to have a well-developed logical framework with concrete objectives and targets and with a set of Key Performance Indicators to monitor achievement of objectives and the resources that are invested. Aspects related to implementation, programme design, monitoring and evaluation system will be streamlined and harmonised at a later stage across initiatives to ensure compliance with the implementation criteria, comparability across initiatives and to simplify the overall landscape.

In case you would like to receive further information about this initiative, please contact:

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[1] https://www.era-learn.eu/documents/final_report_ms_partnerships.pdf

Content

1 General information

1.1 Draft title of the European Partnership

European Partnership for Personalised Medicine (EP PerMed)

1.2 Lead entity (main contact)

DLR is a non-governmental, non-profit German research organisation. Its Project Management Agency (DLR-PT) is mandated by the Federal Ministry of Education and Research (BMBF), the Federal Ministry of Health (BMG) and other ministries to implement and manage governmental programmes for research funding. Being a trustee for the funding budget, DLR-PT administrates, with a staff of over 1.000, more than 2 billion € per year. Areas covered – amongst others – are life sciences, information technology, education and environment. Within the life sciences, the "health" department of PT-DLR manages the areas of clinical and biomedical research for BMBF and BMG in several governmental programmes aiming at fostering research infrastructure as well as supporting internationally competitive research. The health department also hosts the National Contact Point Health and manages funding measures the innovation fund of the Federal Joint Committee (G-BA). DLR is involved in many JPIs and ERA-NET initiatives. For example, DLR coordinates Neuron, HDHL-INTIMIC or ERA-CVD and is beneficiary in ERA PerMed, EJP Rare Diseases, JPcofuND, ERA-HDHL, JPI AMR as well as EDCTP under the H2020 program.

In the context of personalised medicine, DLR was instrumental in establishing ICPerMed and is presently coordinating the ICPerMed Secretariat as well as SINO-EU PerMed (focused on widening Sino-EU policy and research cooperation in Personalised Medicine). DLR is also partner and vice-chair in ERA PerMed as well as a partner in the following ICPerMed-related CSAs:

- Healthcare- and pharma-economics in support of the International Consortium for Personalised Medicine – ICPerMed (HEcoPerMed)
- Personalised Medicine Trials (PERMIT)
- Widening EU-CELAC policy and research cooperation in personalised medicine (EULAC-PerMed)

DLR is implementing the German national action plan "Individualised Medicine" for the BMBF with a budget over 360 Mio €. Currently, the health department also manages a call aiming for the translation of PM with a budget of 50 million €.

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1.4 Summary

Personalised medicine (PM) optimises individual health and health systems. EP PerMed will align PM priorities, provide investments of scale, and create synergies to accelerate PM R&I and implementation. To realise the full potential of this partnership, countries will join forces with the EC to fund transnational consortia, share evidence, demonstrate solutions and to drive supportive activities in policy, regulatory science and health economics. It will collaborate with other health partnerships and reach out to national, regional, and international initiatives and frameworks.

1.4.1 Extended Summary

Personalised medicine is revolutionising biomedical and clinical research while improving the ways healthcare is delivered. The objective of personalised medicine is to deliver the right diagnosis together with the most efficient treatment tailored to an individual patient. In addition, PM can support preventive measures for sub-groups and individuals on the basis of the best available methods and data. Healthcare expenditure is increasing rapidly, and societies also need to minimise the use of ineffective treatments and impact of adverse effects. Furthermore, societies need to enable more healthy and productive years for their populations. PM research encompasses improving our understanding of diseases, and developing innovative therapies, diagnostics and preventive strategies. Thus, PM is a central health concept to develop and provide benefits to patients and citizens. PM will improve health outcomes in sustainable health systems, but also generate new and interesting opportunities for industry and small & medium enterprises in a wide range of fields across Europe and beyond.

Over the next decade, EP PerMed will extensively invest in the discovery and development of innovative personalised approaches and their efficient translation into clinical practice. To maximise potential for adoption, scaling of approaches for broader use will be supported to give fair access to PM for all. Furthermore, the partnership and its members will contribute to these efforts and provide strategies, collaborations and recommendations to enable supportive conditions to promote equal access to the right treatment and preventive approach for each patient and citizen, as well as a sustainable European research and innovation (R&I) and health system landscape on all levels. To achieve this collaboration and dialogue the partnership will involve citizens, patients, research communities, regulators, health economists, and industry as well as a range of payers and healthcare providers. The partnership objectives will include financing of transnational biomedical and PM research and funding of demonstration projects. The latter will examine and showcase successful personalised medicine concepts, approaches and solutions. Moreover, policy requirements to foster and implement PM will be addressed, and recommendations formulated. Partnership support for educational programmes will inform and empower people, research communities, educators, healthcare workers, payers and politicians. The expected impact is to improve the health outcomes for patients and citizens, and to realise optimised healthcare and prevention within sustainable health systems. This will be an important contribution to establishing Europe as an attractive partner for international collaborations and investments related to personalised medicine.

This effort is not starting from scratch as existing initiatives provide a strong basis for establishing a successful EP PerMed. It builds on the International Consortium for Personalised Medicine (*ICPerMed*), the *ERA PerMed* and the so called "*ICPerMed Family*", which is a set of European Commission (EC) funded coordination and support actions (CSA) in the context of ICPerMed. This new European Partnership will generate research and results via funded transnational consortia, networks, collaborations and utilise synergies to integrate all necessary expertise to deliver the set objectives. In this way, EP PerMed will become the most significant platform and initiative to connect key stakeholders in the personalised medicine field. Such stakeholders include for example policy actors, universities, research and patient organisations,

healthcare institutions, industry, research infrastructures, public service institutions at different levels as well as civil and research societies. EP PerMed is strongly dedicated to engaging and cooperating with other European partnerships on health topics, PM-related initiatives and institutions as well as international and regional partners to foster global health and equity of care.

Detailed priorities and activities will be outlined in the Strategic Research and Innovation Agenda (PM SRIA) and the annual work programmes to ensure the relevance of EP PerMed activities and to realise the vision of EP PerMed for the benefit of Europe's patients, citizens and society at large. The PM SRIA is already under preparation with the 1st draft version is planned to be ready by summer 2022. In order to integrate the different PM perspectives, there are several activities foreseen on different levels and sectors related to PM with experts, stakeholders, other partnerships and initiatives as well as the European public. It is expected that the draft will continue to evolve on the basis of the information gathered by the involved Member States, the EC, interested regions and international players. The 1st draft of the PM SRIA will be adapted and finalised in the second half of 2022 and will be a crucial part of the proposal and basis for the annual work plans of EP PerMed.

2 Context, objectives and expected impacts

2.1 Context and problem definition

Personalised Medicine has been established as a key concept for providing tailor-made prevention, diagnosis and treatment strategies for individuals or groups of individuals. PM is a major opportunity for society to provide benefits to patients, citizens and healthcare systems in a framework of equal capacity and fair access to diagnostics and treatments. A growing body of knowledge and technological developments is driving progress in PM and opens up new opportunities for innovation, and better health and care outcomes. This will be beneficial to society and will in parallel ensure the sustainability of the healthcare systems.

The adoption of PM induces a transformative change with immense impact on society at large and the citizens in particular. It demands significant efforts, not the least within R&I, but also to create the right conditions that favour the adoption of PM and ensure access for all citizens to the most advanced and best healthcare solutions, guaranteeing the right treatment for the right person at the right time based on the characterisation of individual phenotypes and genotypes.[2] This characterisation also allows to determine the predisposition to disease and/or to deliver timely and targeted prevention.

To realise the full potential of PM, a European partnership is timely and adequate to address the needs for R&I as well as to establish the required policy and facilitating frameworks. PM represents a shift from a "one size fits all" approach for diagnosis and treatment to one that uses emerging approaches for earlier and more precise diagnosis, to select personalised therapies that achieve the best outcomes or to predict predisposition to disease. The European Partnership for Personalised Medicine, EP PerMed, builds on the foundations laid in 2010-2011 with several preparatory workshops organised by the European Commission, which were followed by a concluding conference in 2011 that served as input for Horizon 2020.[3] The discussions in these meetings eventually led to the Council conclusions[2] (2015) on Personalised Medicine, which

[2] https://data.consilium.europa.eu/doc/document/ST-15054-2015-INIT/en/pdf

[3] https://www.researchgate.net/publication/261441805_Predictive_Preventive_and_Personalised_Medicine_as_the_hardcore_of_%27Horizon_2020%27_EPMA_position_paper/figures?lo=1

provides the current definition of PM which is followed as the basis for EP PerMed.

Another key milestone resulting from the preparatory work was the establishment of ICPerMed in 2016 and the start of the supportive CSA ICPerMed Secretariat. The proposal for EP PerMed builds upon the legacy of ICPerMed and the supporting ERA-Net, ERA PerMed, which was established in 2017 and is based on the CSA PerMed SRIA from 2015.

ERA PerMed, the biggest ERA-Net in the health sector, has engaged 42 funding organisations to participate in different joint transnational calls, including regional and international funders of 32 countries and five continents. ERA PerMed implemented a research agenda according to the ICPerMed Action Plan (2017), through annual joint transnational calls (JTC) for research proposals. With an EC contribution of 9,5 Mio€ and through four calls, ERA PerMed has already successfully achieved to fund 87 transnational research projects in the field of PM, with an investment of over 100 Mio€.[4] This represents a leverage factor of more than 10 for the EC contribution.

ICPerMed currently brings together 46 public and private 'not-for-profit' health research funding and policy organisations from 30 countries and seven regions from four continents.

In 2019, ICPerMed published a "Vision for Personalised Medicine for 2030", based on the input from a broad range of experts throughout Europe. Several key aspects and areas to tackle were identified to accelerate PM in the following ten years:

- further research and robust evidence for personalised medicine,
- educational and training programs for citizens, healthcare professionals and providers,
- transnational cooperation and cross-border exchanges and interoperability for data,
- voluntary public policies and a political agenda across Europe developing new common regulations, definitions of concepts and guidelines,
- innovative systems for data management (collection, storage, access control, sharing) at the EU level and the associated need for new technologies and IT infrastructures,
- massive investments and new financing (or reimbursement) models for PM in healthcare systems across Europe.

To address some specific challenges that might impede the development and adoption of PM, several additional coordination and support actions (CSAs) were established to support the overall agenda of ICPerMed:

- The role and potential added value of regions and regional actors, and the challenges for integrating the needs of remote and sparsely-populated regions and regions with limited innovation and adoption capacities are addressed by the CSAs Regions4PerMed and SAPHIRe.
- The CSA HEcoPerMed addresses the economic models to underpin the adoption of PM in healthcare systems.
- The CSA PERMIT addresses questions related to PM clinical studies and trial design.
- A series of other CSAs address international collaboration in PM with Caribbean and Latin American countries (EULAC PerMed), China (SINO-EU PerMed and

[4] https://erapermed.isciii.es/wp-content/uploads/2021/12/ERA-PerMed-JTC2022-Infoday_CN_09.12.2021.pptx.pdf

IC2PerMed) and Africa (EU-Africa PerMed).

The combined input of these CSAs will contribute to the EP PerMed impact and support the transitional change in healthcare. Thus, major R&I efforts are required to increase the understanding of specific disease mechanisms at a molecular level in order to develop novel therapeutic interventions or provide preventive strategies. Furthermore, the need to establish adequate PM supporting frameworks will be tackled. In parallel, policy adaptions will be sought to ensure access for all citizens in Europe to advanced interventions and prevention strategies based on PM research. These efforts will also contain demonstration and pilot projects to gain hands-on expertise and assessment of the added value of PM especially in the European healthcare systems.

Over the years, the science basis for PM has been evolving from demonstrating the effectiveness of personalised diagnosis and treatment of some rare diseases and cancers to the possibility of using biomarkers to predict any individual's disease risk and thus prevent the onset of common complex diseases. It is clear that the potential of PM goes beyond treatment to include prevention, health promotion, and disease management. The increased understanding of biological, lifestyle and environmental factors that regulate disease onset and disease progression, together with the prolific technological advancements for the discovery and detection of biomarkers in the last two decades will drive progress in discoveries supporting PM.

Implementation of PM in healthcare systems for the benefit of the citizen is still at an early stage, involving a multitude of considerations beyond biomedical ones. Conceptually, PM may be seen as a natural evolution of medicine. However, in practical terms it may well represent a major disruption for healthcare systems, implying a shift from public health concepts traditionally developed for populations to a focus on the individual. Thus, reforms in healthcare systems are necessary to accommodate this change in paradigm.

A lot has already been achieved in many PM areas, while progress in other crucial areas is still very slow. This partnership will bring together substantial funding for research and innovation projects within PM and create an effective platform for sharing of information, evidence, policies, and know-how. It thereby strongly addresses the challenge to "leave no one behind".

2.2 Common vision, objectives and expected impacts

The vision of the European Partnership for Personalised Medicine is to improve health outcomes within sustainable healthcare systems through research, and the development and implementation of personalised medicine approaches for the benefit of patients, citizens and society.

PM is a shift from a "one size fits all" approach for diagnosis and treatment to one that uses emergent approaches for earlier and more precise diagnosis, to predict predisposition to disease or to select personalised therapies that achieve the best outcomes.

To realise this vision, EP PerMed will aim to integrate all the elements needed in a collaborative effort to achieve the full potential of PM. The partnership is focusing both on joint research and innovation actions, and activities to ensure appropriate supportive conditions such as policy and regulatory frameworks, end-user involvement, education and knowledge exchange, aligned strategies and priority settings. The partnership will work through its own activities as well as close collaborations with other initiatives to actively contribute to all these elements. The members and associated partners of EP PerMed will drive the activities with support and input

from a broad network of stakeholders. The expected impacts (fig. 1) are to improve the health outcomes for citizens and patients, contribute to sustainable health systems and position Europe as an attractive partner for international collaborations and investments in the field of PM.

EP PerMed will contribute to the following **general objectives**:

- Improved health outcomes for citizens and patients,
- Optimised and sustainable healthcare,
- Europe at the forefront of research and innovation, in collaboration with international partners.

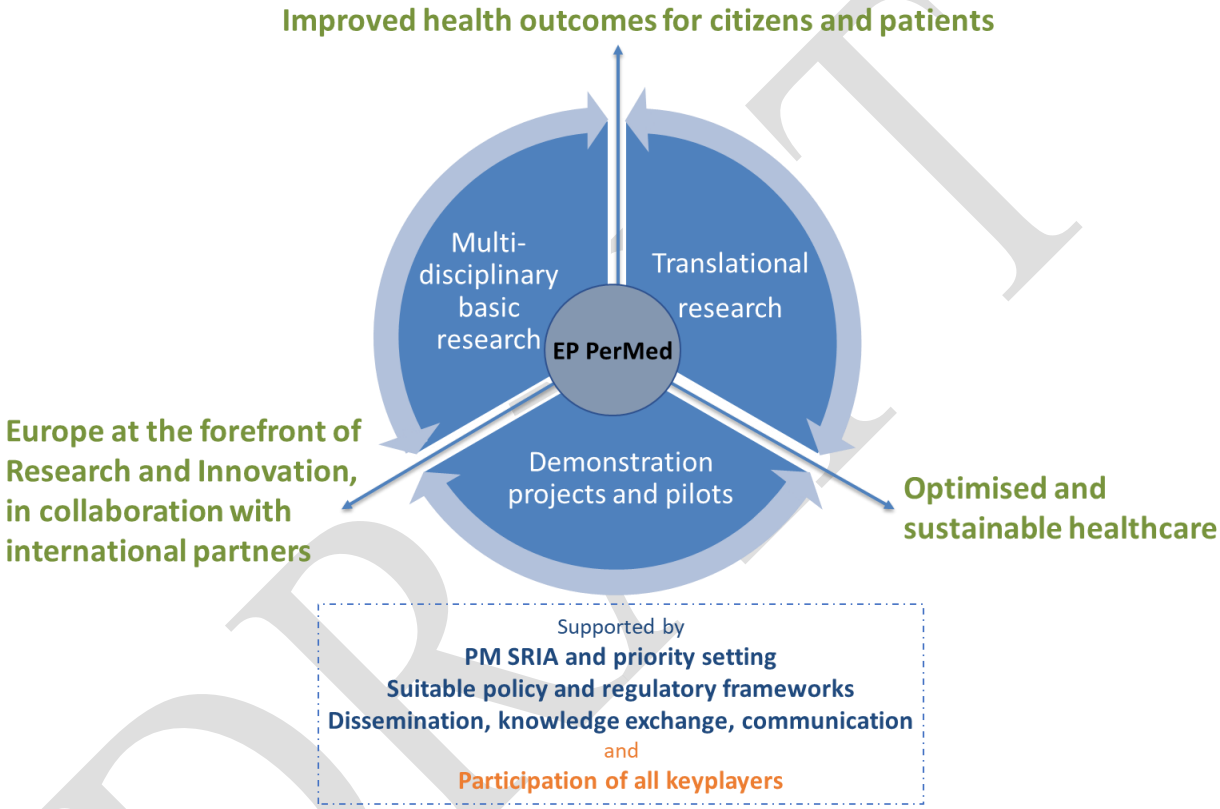

Figure 1: The EP PerMed vision

Personalised medicine will contribute to the general objectives through the following **specific objectives**:

- To deliver novel research results, insights and solutions based on innovative PM approaches, including personalised preventive measures.
- To accelerate the uptake of innovative PM approaches in healthcare systems.
- To promote the translation of basic PM research results into clinical practice and upscale to market access.
- To promote business models/transfer to the market.
- To promote access to PM approaches for patients, citizens, and society at large.
- To ensure EU-wide access to the most advanced PM-based interventions for all. This includes, e.g. needs in the regulatory and legislation field, health economic aspects, measures to attract public and private investments and development of ethical frameworks.

- To accelerate deployment, such as innovation procurement, financial support mechanisms for upscaling of pilot initiatives and an investment portfolio to attract public and private investments.
- To ensure appropriate supporting conditions to realise the full insight and potential of PM approaches.
- To enhance the skills of the relevant PM workforce, and improve citizen awareness as well as enable capacity-building across Europe.
- To establish a highly connected PM R&I system comprising a network of national and regional knowledge hubs for PM, throughout Europe.

EP PerMed will contribute substantially to the specific objectives through dedicated partnership specific **operational objectives**. The operational objectives will be translated into activities described in section 3. These activities will be further developed together with the future members of the EP PerMed during 2022. The objectives will be monitored and evaluated throughout the course of the partnership using appropriate impact measures.

The EP PerMed operational objectives are:

1. Maximise synergies and align priority setting in R&I

The organisations joining the EP PerMed will agree on a common Personalised Medicine Strategic and Innovation Agenda (PM SRIA). The PM SRIA will be the basis to identify research and innovation (R&I) priorities that will accelerate and maximise the impact of PM.
The SRIA will be implemented through Annual Work Plans to support the translation of the priorities identified by the EP PerMed consortium and the member states into partnership activities. The PM SRIA and the first annual workplan will be available at the start of the partnership. The PM SRIA will build on the previous work across a range of initiatives on a European and national level, including the ICPerMed family, and ERA PerMed and related initiatives.

The Annual Work Plans will elaborate the procedures and tools to implement the SRIA, i.e. the calls and evaluation procedures, the topics and priorities addressed by the joint transnational calls as well as the activities aiming at developing the necessary frameworks, policies, and activities accelerating the uptake of innovative PM approaches. The partnership will continuously monitor the implementation of the PM SRIA and refresh the SRIA as appropriate, to ensure it reflects international scientific developments in the field of PM, in line with the outcomes of EP PerMed supported projects and in accordance with the implementation over the duration of the partnership.

The partnership will support the establishment and operation of national mirror groups in the health sector or national hubs for PM[5] in participating countries. PM hubs will establish a think tank fostering the implementation of PM and strengthening the PM community within one country. They will allow alignment of strategies as well as dissemination and exploitation activities within one country increasing transparency and sustainability. PM hubs allow countries to provide input and contribute actively to the different EP PerMed activities and act as the link to national/regional RD programmes, infrastructures, centres of expertise and strategies. Hubs can interface with hubs from other countries, share experiences and information and leverage incentives, which will be of particular importance for underrepresented countries. Pooling of different national and regional resources will maximise the impact of investments in research programmes and healthcare systems

[5] https://www.icpermed.eu/media/content/EPPerMed_InfoSheet3%20-%20National_Hubs_for_PM.pdf

2. Increase investment, expand the knowledge base and foster the development of new innovative PM approaches

The partnership will work with a broad range of stakeholders from European, national, regional and international initiatives, to fine tune R&I priorities along the whole value chain. To realise the full potential of PM, the knowledge base and innovative approaches will be further expanded across the entire value chain.

The PM SRIA will be implemented through joint transnational calls fostering multidisciplinary and intersectoral research, including academia, clinics as well as primary healthcare providers and industry as well as NGOs. Research activities will focus on supporting the PM science base; on translating new insights into innovative diagnostics, therapies, interventions and prevention strategies; and on testing and demonstrating such innovations by end-users in healthcare settings. This could also include the development of research supporting requirements, e.g. data registries and analytical pipelines as well as reflections on their uptake in regional/national health systems. Specifically addressing healthcare, regulatory and health economic needs will be encouraged within supported R&I projects.

3. Accelerating the absorption and implementation of PM

EP PerMed will emphasise the acceleration of the adoption of PM by users and its uptake into healthcare systems. A package of dedicated activities will be developed by the partnership to achieve this. It will include mapping of the outcomes of projects that received funding through ERA PerMed and projects funded through the partnership to identify opportunities for PM implementation in healthcare settings. A dedicated set of measures supporting the trajectory from research findings to implementation will be envisioned to stimulate the technology transfer.

R&I projects will be encouraged to consider the potential for technology transfer in their projects from the onset. An active portfolio management approach and innovation acceleration measures will be developed (for example through a network of technology transfer offices, TTOs) to support the translation of novel insights into innovative applications and to maximise the chance of these applications to reach the market and the end users. This Accelerator will enable the incubation of newly identified projects until market readiness and will help to attract public and private funding. The EP PerMed Accelerator may also reserve budgets for patenting findings from EP PerMed funded projects.

The partnership will furthermore promote collaborations that foster the translation from research to market access and healthcare system implementation. To accelerate the uptake of PM approaches by end-users, novel or tailored business models will be supported and tested in demonstration projects. In this respect, the EP PerMed will exchange, communicate and collaborate with other initiatives and partnerships, such as IHI-JU, EJP Rare Diseases (future EP Rare diseases) and EP THCS.

4. Contribute to necessary frameworks, policies and activities for implementing PM approaches

Apart from the R&I activities of EP PerMed, the annual work plans will also address the development of suitable framework conditions in support of the approaches. Appropriate policy, regulatory and financial frameworks are essential to accelerate the development and uptake of PM diagnostics, treatments and prevention across Europe, while driving equal access to the most

efficient therapies and interventions. Mechanisms will be developed to advance innovative projects until they become self-supportive or novel insights are transferred into the market. Suitable financial options need to be established to accompany and support these actions.

The partnerships annual work plans will also address identified policy needs to enable the broader adoption of specific PM approaches. Emphasis will be given to address end-user needs including the requirements of the healthcare sector, citizens and industry to develop and adopt PM-based approaches. Through strategic position papers and other activities, EP PerMed will create awareness, initiate discussions, and provide recommendations to advance the PM agenda. Several of the policy aspects will be associated to **regulatory considerations, appropriate legal and ethical frameworks** as well as **reimbursement models** for PM implementation. In this respect both the demonstration of added value of PM approaches for patients and citizens and the context of the long-term sustainability of healthcare systems have to be assessed (e.g. via health technology assessment, HTA).

To promote and foster access to PM for all, EP PerMed will communicate with R&I organisations and healthcare systems across Europe as these are often under different authority levels in different countries. It is essential to ensure that not only national authorities are reached, but also relevant regional authorities. Thus, the partnership will develop participation options to ensure regions can join the discussion process and joint activities. This will correspondingly encourage the mobilisation of regional funds for EP PerMed joint transnational calls and further activities. The operational objectives will be realised through dedicated actions.

5. Education, knowledge exchange and creating awareness

The partnership will build on results and experience from a range of European and national initiatives, in particular ERA PerMed, ICPerMed and its CSA family, the 1+MG project and related initiatives. EP PerMed activities will include a solid communication and dissemination plan building on previous work within the ICPerMed family, including a stakeholder forum and close interactions with related initiatives (i.e. other European partnerships), to foster discussions on relevant PM topics and strategic reflections. The PM community will be interlinked and the wider dissemination of PM ensured through dedicated events such as workshops, conferences, and other forms of meetings. Outputs from these exchanges will feed into strategic documents and a PM-related priority setting. The communication and dissemination activities will create awareness for PM and its potential among healthcare professionals and citizens, as well as provide targeted input to decision-makers, payers and regulators. This will for example be done through the preparation of "position papers". These documents will examine the potential of PM-based approaches for prevention, diagnosis, monitoring and treatment, promote good practices and standards for PM implementation, and will provide analysis in relation to health outcomes and cost-effectiveness as well as other challenges.

Ethical, Legal, and Social Implications (ELSI) of PM will inform targeted literacy efforts to support citizen and patient empowerment and engagement. Above all ELSI considerations throughout all relevant EP PerMed activities should build and maintain trust as this is a crucial requirement to develop and implement PM. The development of PM hinges on the analysis of huge amounts of health-, omics- and life style data from citizens. Without a guarantee of correct use of such data, appropriate safeguards, and solid protection of privacy, citizens will not agree to sharing their data. In this respect, it is critical that the issue of building trust is of paramount importance, as lack of trust will hamper citizens participation which is absolutely essential for this endeavour. The ELSI framework will interface with and be informed by relevant regional, national and European initiatives, including the 1+MG initiative.

An increased awareness by the public and healthcare providers will drive the demand to decision makers for equal and fair access to novel PM approaches. An increased awareness will also stimulate regulators and payers to address the needs for the implementation of novel PM approaches to be implemented in the healthcare systems. This should expand access to PM and related technologies for all. The work of HEcoPerMed will be a crucial aspect for these purposes. In addition, the maturity models developed by 1+MG may be tested in different countries and regions under EP PerMed. This should allow provision of more tailored recommendations to these regions and countries on how to accelerate the adoption of PM. EP PerMed envisions the need to support projects focused on PM implementation based on such recommendations.

The communication and dissemination actions will also emphasise the needs for education and life-long training strategies for stakeholders in order to develop jobs and skills for the future while embracing PM to the fullest extent in current healthcare practice. To name one example: PM is very data and technology driven, there are growing and evolving education and training needs for healthcare professionals. Networking activities under EP PerMed may facilitate the development of standards and common approaches across partners (countries or institutions), ensuring shared knowledge, data and exchange of good practice for the benefit of citizens and patients.

The catalogue of best practice examples of PM implementation will be maintained and developed further with information extracted from the partnership's activities as this has proven to be beneficial, e.g. for ICPerMed members. Finally, EP PerMed aims to be the main source of information and news on PM advances in Europe for citizens and decision makers. This would cover all aspects of PM, including, but not limited to ELSI frameworks, regulatory needs, cost-effectiveness & reimbursement models supporting the adoption of PM in healthcare systems, and guidelines to implement PM.

2.2.1 Links and Collaborations (HORIZON 2020, HORIZON EUROPE and other)

Information and outputs from the ICPerMed family will be used as a solid basis for development of e.g. the PM SRIA and the annual work plan of EP PerMed. In particular, the work of PERMIT, a consortium focussing on clinical studies in PM and HEcoPerMed will be valuable for the future work of EP PerMed. The CSAs focusing on regional involvement provide valuable input for understanding regional specificities and assets, as do the CSAs concentrating on international collaboration. Also, it will be important to engage with planned new CSA activities such as the one on personalised prevention and build synergies.

EP PerMed will also reach out to its fellow partnerships under cluster 1 Health of Horizon Europe. In particular, the European Partnership on Transforming Healthcare Systems (THCS) is expected to be key to support the broader adoption of PM in healthcare systems across Europe. The EP Rare diseases will be an important partner to align with and potentially join forces as this partnership may be considered a pioneer for acknowledging and demonstrating the strength of PM. The partnership ERA4Health is also an interesting partner.

Interaction and alignment with the Innovative Health Initiative Joint Undertaking (IHI JU) will be valuable to support industry engagement with EP PerMed. IHI JU is the follow up initiative of IMI2 JU, which had a strong focus on PM. Under IHI JU, data and medical technologies industries are also represented. The interaction with IHI JU will help to support clinical studies and bring translational research a step further, to facilitate knowledge transfer and market access for developed solutions. The latter may be supported through an interaction and alignment with the ERA4Health which will, for example, include a special focus on researcher initiated clinical

studies.

To accelerate the translation of biomedical and other research results to clinical research the interaction with research infrastructures, such as EU Openscreen in screening drug efficacy on patient cells & tissue in a personalised manner, BBMRI, EATRIS and ECRIN will be valuable assets. Alignment with regional initiatives, as the Vanguard Initiative Pilot Smart Health, will be sought to support the involvement with European regions and industry involvement as well as the uptake of PM.

The optimisation of framework conditions will also rely on close communication and interactions with the One Million Genomes Initiative and the future development of European federated infrastructures for genomics and cancer images as well as the development of a European Health Data Space. The achievements of these initiatives will foster the harmonisation and interoperability of data which is a prerequisite for gaining the full potential of PM. In turn, the objectives of EP PerMed will provide a platform for the development and translation of data into knowledge and innovative approaches.

To advance successfully with PM implementation and complement the work proposed with other features of the European research landscape, EP PerMed will also seek synergies with other partnerships such "One Health/AMR" and "Pandemic Preparedness and Societal Resilience", and other Health Programmes, including the Mission on cancer and initiatives as the "Global Alliance for Genomics and Health (GA4GH)".

EP PerMed will accelerate the development and uptake of PM throughout Europe. This will ultimately contribute to improved wellbeing and to the sustainability of health systems. Through providing the right therapy for the right person at the right time, overuse of medication can be controlled, and adverse effects minimised while also increasing patient's quality of life. In an indirect manner this will contribute to resource efficiency and hence to the ambitions of the Green Deal and the principles of Do Not Significant Harm (DNSH). The partnership will also contribute to the Data Strategy (EU Health Data Space), the Pharmaceutical Strategy for Europe, the Digital Europe Programme, EU4 Health as well as HERA initiative and the Beating Cancer action plan, including the Mission on cancer.

2.2.2 Expected Outcome and Impact

The operational objectives will be supported by specific activities described in section 3. These will ensure EP PerMed will realise significant outputs and contribute to the expected outcomes and impacts. The intervention logic (fig. 2) summarises how the operational objectives will contribute to the overall impact.

Outputs:

Through the implementation of the SRIA, EP PerMed will align priorities, generate an impactful project portfolio, yielding research publications and position papers as a result of these projects. The research papers will demonstrate the increase of new scientific knowledge that eventually will lead to innovative PM approaches for diagnosis, treatment, and prevention.

As outputs from the operational objective 4 to develop supporting framework conditions, and objective 3 to accelerate the absorption of PM, the innovative opportunities of PM adoption in healthcare systems will be aligned with national and regional priorities. EP PerMed will deliver options for appropriate policy and regulatory frameworks and sustainable business models to implement PM in healthcare systems.

Outcomes:
The activities under EP PerMed will deliver innovative PM approaches for diagnosis, treatment, and prevention, and maximise uptake of PM in national or regional healthcare policies. EP PerMed will proactively contribute to the translation of new scientific insights into clinical practice and transfer of R&I to the market. This will be supported by the development of innovative business models to ensure accelerated uptake of PM in healthcare systems. EP PerMed will also deliver the evidence base for the health, social, and cost benefits and efficiency of PM. An important outcome of the partnership will be the establishment of a highly connected PM R&I ecosystem comprising a network of national and regional knowledge hubs for PM throughout Europe.

Impact:
The long-term aim of the partnership is to ensure lasting impact at multiple levels. EP PerMed will put Europe at the forefront of R&I in PM development, uptake and implementation. For society and the citizens, it will promote access for all citizens to the most advanced therapies and interventions, including prevention approaches based on personal and dedicated characterisation for improved health outcomes. The actions of the partnership will stimulate countries to more quickly embrace the potential offered by PM, which will in turn lead to optimised and sustainable healthcare based on improved knowledge and understanding of the health and cost benefits of PM.

Focused investment in PM research, innovation and healthcare will also strengthen the European Research Area as well as the innovative and competitive landscape with new employment based on the development of PM, also in collaboration with international partners.

The partnership will develop a series of activities along the general and specific objectives, summarised in figure 2. Thus, the EP PerMed strategy will in general achieve the expected outcomes and impact of PM on the global health and specifically on improved health outcomes and Europe's position in R&I.

The European Partnership for Personalised Medicine Vision:

Improve health outcomes within sustainable healthcare systems through research, and the development and implementation of personalised medicine approaches for the benefit of patients, citizens and society.

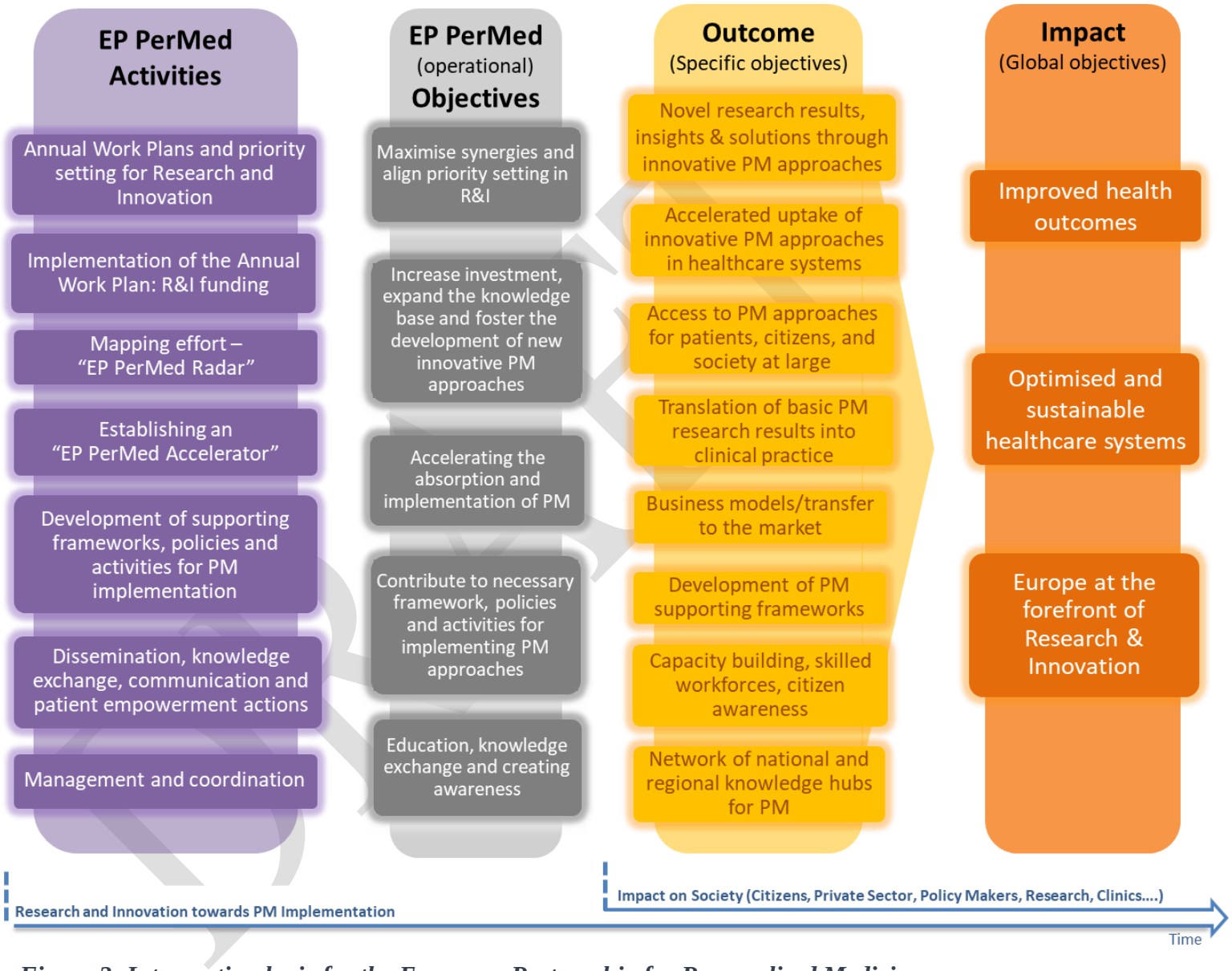

Figure 2: Intervention logic for the European Partnership for Personalised Medicine.

2.3 Necessity for a European Partnership for Personalised Medicine

The promotion of social cohesion and inclusiveness and the health and well-being of its people are central aims of the EU's programmes. These aims permeate the overall political priorities of the EC[6] and in the strategic plan for Horizon Europe[7], in which Cluster 1 will enlarge the range of health-related issues addressed. Cluster 1 will program investments to achieve the following six expected impacts:

- Staying healthy in a rapidly changing society
- Living and working in a health-promoting environment
- Tackling diseases and reducing disease burden
- Ensuring access to innovative, sustainable, and high-quality healthcare
- Unlocking the full potential of new tools, technologies, and digital solutions for a healthy society
- Maintaining an innovative, sustainable, and globally competitive health-related industry

PM plays a key role in achieving these impacts, both in terms of delivering new and improved therapies for patients and in terms of providing targeted and effective prevention measures for the people in Europe. For citizens to effectively have access to PM, there are still many obstacles to overcome, both regarding R&I and PM implementation. For example, health economic aspects are growing in importance as implementation activities increase, and new developments and economic models are critically needed. In addition, regional and national differences in the accessibility of diagnosis and care increase the risk of migration of patients and citizens from their own local healthcare system into other regions or countries. This affects the efficiency of healthcare and creates social inequalities in the access to healthcare. Thus, in many areas a lot has already been achieved, while progress in other crucial areas is still very slow.
Cooperation and investment across Europe as well as internationally are necessary to ensure the continued progress of PM development and implementation. A large number of initiatives are addressing various parts of these needs (e.g. H2020 programmes, individual regional and national programmes). **However, EP PerMed is the most significant platform which will foster cooperation and networking among all key stakeholders throughout the full value chain across Europe and beyond, both from a funding as well as a policy perspective**.

Based on a common aligned PM strategy, EP PerMed will enable PM R&I, capacity-building, creation of synergies, high level policy discussions, and the development of recommendations and adapted or new standards such as for data sharing, harmonisation, interoperability or for *in silico* models. The partnership will ensure that drivers of development support equity across European countries and regions. As a result, the partnership will be the most significant and central initiative to effectively promote at all levels PM-related efforts for the benefit of people and society. EP PerMed-driven R&I activities will greatly support Europe's position at the forefront of:

- PM science base
- Personalised therapy, diagnosis and prevention strategies
- Strategies for the uptake of PM innovation by healthcare systems for improved health outcomes and optimised and sustainable healthcare
- Strengthened innovative and competitive landscape with new employments in PM
- Improved knowledge and understanding of the health and economic benefits of PM.

[6] https://ec.europa.eu/info/strategy/priorities-2019-2024_en https://ec.europa.eu/info/strategy/priorities-2019-2024_en https://ec.europa.eu/info/strategy/priorities-2019-2024_en https://ec.europa.eu/info/strategy/priorities-2019-2024_en
[7] https://ec.europa.eu/info/research-and-innovation/funding/funding-opportunities/funding-programmes-and-open-calls/horizon-europe/strategic-plan_en

EP PerMed is building on a range of existing and past activities and achievements, and the long-term sustainability is important from different perspectives:

- The multitude of PM-related activities under Horizon 2020 has created a wealth of results, networks, and knowledge. EP PerMed will ensure that Europe capitalises on these efforts to push PM-related activities forward. Strategic documents developed by the diverse initiatives will inform the PM SRIA and many actors of those projects will actively join the partnership as members or associated partners, or through the stakeholder group.
- In particular, ICPerMed, ERA PerMed and the ICPerMed Family have over the last 6 years been instrumental in promoting PM through joint research funding, dissemination, communications, events and policy activities. It is expected that the value created will be taken up and used by EP PerMed to the maximum extent in the further promotion of PM.
- By the end of the Horizon Europe framework programme and the partnership lifespan, extensive, novel value will have been created. EP PerMed will ensure the continuity and sustainability of activities, e.g. towards a European Institute for Personalised Medicine, through significant investments in R&I, active portfolio management, an appropriate supportive framework, continuous monitoring of the state of the art of PM and reassessing and refreshing the PM SRIA, as needed.

Overall, EP PerMed will be the central Member States and EC initiative to foster cooperation and networking among the relevant stakeholders for PM, both from a funding as well as a policy perspective. The new partnership will thereby effectively enable the different participating countries and regions to implement PM approaches to the benefit of citizens and patients, thereby supporting the objectives of Horizon Europe and common political priorities of the EU and its Member States.

2.4 Partner composition and target groups

The EP PerMed will identify the requirements for a suitable PM framework in terms of infrastructures, resources, and regulatory procedures to foster the development and implementation in an efficient and multinational coordinated approach aiming at avoiding duplications and allowing the inclusion of all different countries in the process, independently from their progress and achievements in this field. The diversity of the national and regional healthcare systems and funding structures makes convergence difficult, but the partnership is inclusive and encourages participation from all European countries as well as international organisations from different continents, including countries with small research communities and different advancements in PM. The integration of ICPerMed in the partnership is foreseen and will support the international dimension and inclusion of organisations from all over the globe, e.g. from third countries, not associated to Horizon Europe. This allows in the first line Europe to fulfil its aim to “leave no one behind”. It provides the needed support for the PM end-users, including patients and citizens, in the form of healthcare independent of their country of origin or social status. It allows generalists and other prescribers to better understand and apply PM approaches. Thus, EP PerMed will contribute to improved health outcomes through PM approaches for the benefit of patients, citizens, and society as a whole.

The high-level participation, in form of ministries and funding organisations, allows multinational funding activities with high and sustainable commitments (see also section 3). Furthermore, the alignment of strategies (political priorities) amongst these high-level organisations is key to translate these efforts into practice in a coordinated way, even across borders. Through sharing knowledge and joining forces, this collaboration can promote the best

diagnosis and treatment options for patients, and move towards prevention for a healthy society. The partnership connects national activities and programmes and actively incorporates regions. Strategic documents developed by EP PerMed will facilitate consensus-building and decision-making processes in participating organisations and countries, especially EU Member States, and thereby enable more coordinated and impactful actions in the PM field.

2.4.1 Stakeholder group

EP PerMed provides a major opportunity to bring together all stakeholders needed for effective PM research, market access and implementation in the health systems. Existing networks from the ICPerMed, ERA PerMed and the ICPerMed Family provide the basis for building this partnership, which creates the synergies needed for the integration of all essential expertise in PM from the stakeholder community. These different actions already demonstrate that real progress in PM can only be achieved when research and implementation efforts include the entire value chain. This was documented in the PerMed Strategic Research and Innovation Agenda (2015), describing challenges and giving recommendations for advancing the PM field, the ICPerMed Action Plan, defining "actionable research items" to be addressed through research funding, and the ICPerMed Vision Paper outlining how the use of PM approaches will promote "next-generation" medicine in 2030.

Through stakeholder groups, the EP PerMed will be in close and regular contact with all these interested parties. Stakeholders will be informed about the work of the partnership, invited to workshops, conferences or partnership meetings and solicited by the different pillars leads to be part of the work and to contribute to the goals and achievements of the partnership. The stakeholder community will be involved from the outset of the partnership and will be crucial in supporting the successful development and implementation of PM approaches. They will contribute to the identification of challenges, and opportunities for alignment at regional, national, European and international levels with complementary access to resources, information and data. The benefits of participation for different stakeholders include:

- Researchers – enhance collaborative PM research; achieve transnational collaborations; consolidate research beyond the EU; sharing of results by Open Science principles;
- Health and care authorities, policymakers and other stakeholders – development of evidence-based PM strategies and policies; sharing good PM practices; strategic alignments; increasing of resources; efficient investments in PM;
- Citizens, health and care professionals – increase PM literacy; identifying and prioritising PM needs; better PM understanding and application;
- Healthcare providers and professionals – increase knowledge on PM implementation;
- Innovators and local/regional private stakeholders – joining forces to facilitate PM uptake of successful innovations;
- Patients, citizens and society as whole – engagement in PM implementation.

Ideally, the cooperation of all stakeholders will facilitate consensus-building and decision-making processes in the implementation of PM research outcomes into the health systems with respect to current and future needs.

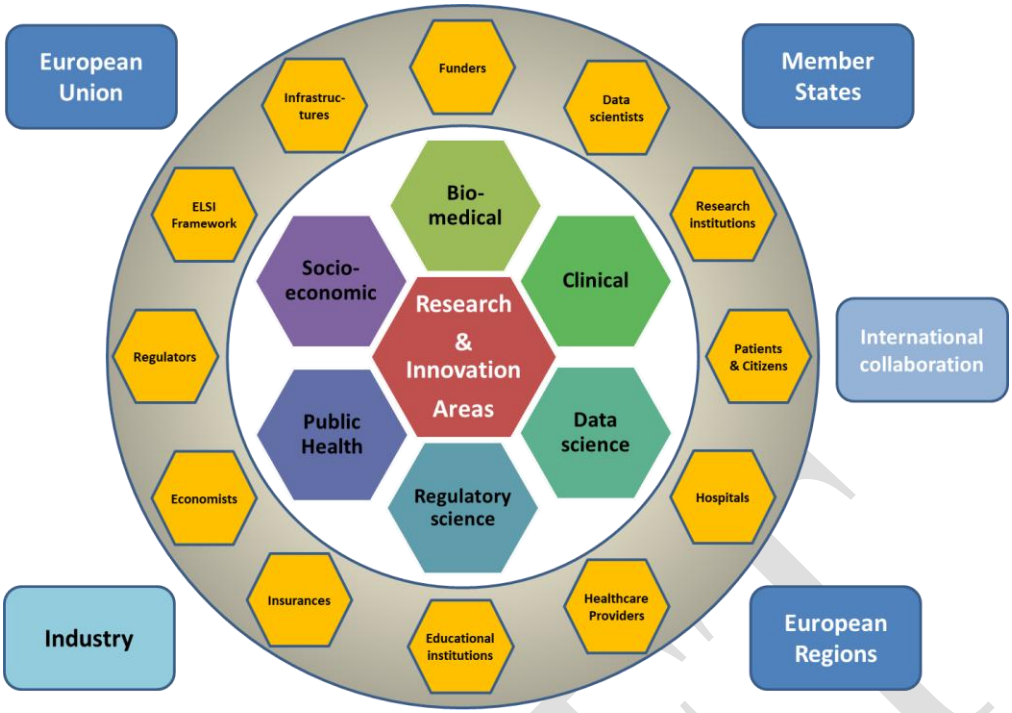

Figure 3: The personalised medicine stakeholder community.

2.4.2 International collaboration

Considering that PM is an international effort, with excellent experts and initiatives located around the globe, EP PerMed aims to involve international as well as European organisations, including those based in third countries to the Horizon Europe framework programme.

The past years have demonstrated ample evidence of the willingness for knowledge exchange activities in the field of PM between European Member States and international partners, including the wider distribution of innovative PM practices. Important steps were taken to identify and connect relevant European and international PM communities, e.g. via events, but also through joint research funding activities. This has been shown to be essential, for instance, in attaining a critical mass of data from various sources to develop and train algorithms and models used in PM approaches. Additionally, such joint efforts have proven to be critical in ensuring that developed approaches are applicable as broadly as possible and not limited to a specific regional context, which would limit potential for public good.

Global collaborations to foster strategic engagement, as well as investment in PM will be a core component of EP PerMed and realised through the integration of ICPerMed. The partnership will broaden the structure and interlinks through funding activities, stimulating transnational research and the development of networks. Ultimately, these efforts deployed through specific support activities will foster related policy development and appropriate implementation of PM at an international level. The partnership will be a platform to develop a global PM agenda, integrating and promoting common standards.

3 Planned Implementation

3.1 Activities

EP PerMed will improve health outcomes and optimise healthcare systems by supporting and accelerating PM development and uptake in healthcare. It will position Europe, in collaboration with international partners, at the forefront of R&I in the PM field. To realise its vision, EP PerMed identified five operational objectives (section 2.2) which are translated into concrete activities as follows:

- Developing Annual Work Plans and priority setting for R&I;
- Implementation of EP PerMed R&I funding;
- Establishing the "EP PerMed Radar" – Mapping outcomes of PM R&I;
- Establishing an "EP PerMed Accelerator" to accelerate absorption of PM by users and healthcare systems;
- Contribute to the development of the necessary frameworks, policies and activities for implementing PM approaches;
- Development of a suitable policy and regulatory framework establishment, which is essential for the development and uptake of innovative PM approaches: this will improve the knowledge base and support the development of strategic recommendations and policies for PM implementation in healthcare systems;
- Dissemination, knowledge exchange, communication and patient empowerment actions. Dissemination and communication will address a broad range of activities including contributions to the dissemination of good practice examples, educational programmes, improved literacy, and knowledge exchange: improve the knowledge base, support the development of education programmes and literacy campaigns, create synergies, facilitate communication, Education and Training (E&T) activities;
- Ensure coordination and management of the partnership: The activities are interconnected and coordinated by an overall management structure that will guarantee appropriate governance and programme management and facilitate the information flow and cooperation between the different activities and work streams. The management unit will include the EP PerMed Secretariat.

3.1.1. Annual Work Plans and priority setting for Research and Innovation

To realise the operational objective 1: Maximise synergies and align priority setting in R&I, Annual Work Plans covering the priorities for R&I will be developed.

The partnership will be based on a common personalised medicine strategic and innovation agenda (PM SRIA) aligning European, national, and regional strategic priorities. A roadmap will outline the implementation until the end of the partnership. The PM SRIA will be the basis for the EP PerMed Annual Work Plans and priority setting to foster the policy development, ensure resource allocation and the alignment of research funding strategies. The Annual Work Plans will indicate the priorities which will be addressed for implementation.

3.1.2. Implementation of the EP PerMed Annual Work Plan: R&I funding

To achieve the operational objective 2: Increase investment, expand the knowledge base and foster the development of new innovative PM approaches, EP PerMed will organise various joint funding activities, with topics identified in the PM SRIA and translated in the annual work

plans. Therewith, EP PerMed will align regional and national public investments, invest in transnational research and activities, bring new insights and enable innovative PM applications and ultimately facilitate the uptake of PM approaches in the health systems and by users in general. The partnership envisions not only support for R&I projects, but also networking initiatives, coordination actions in support of demonstration and pilot projects, and dedicated education and training projects. These projects will be selected in line with the identified priorities and in collaboration with the contributing funding agencies to optimise the PM European Research Area.

Funding will be coordinated in calls covering the breadth of the SRIA. In addition, alignment will be sought with other on-going initiatives, including industry driven ones (e.g. IHI JU) to foster the translation of research to the market. Alignment will also be sought with regional initiatives (e.g. the Vanguard Initiative Smart Health Pilot) to align with regional funds that support interregional PM-based projects.

Health data sharing and interoperability will be promoted, as well as the open access and sharing of research data, results and outcomes of the funded projects. The main actors supporting this activity will be public and private funders.

The different calls will be open for joint funding from members and associated partners of EP PerMed as well as other regional and national funders in the field of PM, including third countries to Horizon Europe. Collaboration with international partners is essential to obtain the required critical mass, e.g. in terms of data and expertise, and is key to develop PM approaches that are broadly applicable. The partnership will launch at least one co-funded joint transnational call annually – addressing R&I projects, one call to support networking activities, and one call to support demonstration and pilot projects:

I. Transnational, Multidisciplinary Research Funding (ERA Net-like calls)

Based on successful ERA PerMed experiences, two types of calls for funding transnational research projects in the field of PM will be implemented: A) co-funded calls, with participation of the EC, e.g. via gap filling (EC top-up); and B) non-co-funded calls, organised amongst funding organisations without additional budget from the EC for research project funding. A virtual "common pot" will be used, where each organisation is funding research teams from its own region/country according to individual regional/national specific funding regulations. A two-stage process, with a pre-proposal and full-proposal phase, will allow efficient call management and ongoing funding commitment. Peer review steps in both phases will allow the promotion of scientific excellence and funding of high-quality research projects.

The funding organisations participating in the calls are all engaged in developing the call topics based on the priorities set in the PM SRIA and the action plan/annual work plans of the partnership. As the PM field is broad, a scientific committee will be consulted for refining the call topics, i.e. ad hoc Call Advisory Boards (CAB) for each individual call containing experts with the appropriate expertise for the proposed topic.

II. Networking Calls

Networking calls aim to connect different key players in PM to build synergies and capacities, facilitate sharing of knowledge and infrastructures and enhance resource alignment and maximise existing and future efforts in the field of PM. This type of call will foster the development of white papers, horizon scanning, guidelines and recommendations as well as best practice frameworks in order to identify key questions to be addressed or outlining potential solutions for overcoming barriers connected to PM development and implementation, such as those associated with health data sharing and interoperability. Networking calls will create

synergies in the form of new collaborations for PM knowledge sharing among healthcare professionals including primary healthcare and clinicians, researchers, informatics experts, industry/private sector and patients, to enable or increase multidisciplinary network structures to design and implement potential solutions for supporting PM research. Furthermore, they will bridge the gap from research to market-access via development of innovative collaborations. Through networking calls, the partnership will enable and increase the participation of underrepresented countries in new and existing PM research networks – fostering the participation of EU13 countries in research networks and consequently in future consortia funded via JTCs.

III. Research Innovation and Technology Calls (RITC, demonstration projects and pilots)

Research Innovation and Technology Calls (RITC) are intended to support PM approaches and research outcomes further towards translation to the market, by actively connecting experts in the different PM fields including from the private sector, research, clinics and education to develop the most promising solutions into commercially viable products or services, that could be ready for uptake and implementation.

RITCs will invite participation from funders interested in start-up funding and that can provide funding to higher Technology Readiness Levels (TRL)[8] levels. In addition to public funders, these calls would seek the participation of industry actors as funding partners.

Demonstration projects or pilots can be used to test solutions in real world settings, gathering stakeholders to address these several dimensions: skills, capabilities and cultures, infrastructures, standards, policies and regulation, technologies, products and processes, business models and tenders.

3.1.3. Mapping effort – "EP PerMed Radar"

To realise operational objective 3: Accelerating the absorption and implementation of PM, two dedicated activities will be performed, i.e. mapping of the outcome of EP PerMed funded projects and developing activities to accelerate absorption of PM (section 3.1.4). The partnership will establish an EP PerMed Radar for the early identification of results from R&I projects funded under the partnership and the projects that received funding through the ERA-Net ERA PerMed. Dedicated mapping activities will cover the monitoring, collection, and analysis of information about relevant developments in PM.

The EP PerMed Radar will allow the partnership to follow-up on the implementation of the PM SRIA and annual work plans and to reassess the SRIA on a regular manner, i.e. to adapt, revise and update the SRIA when necessary. The mapping activities will prevent overlapping and duplication with other relevant initiatives of, for example, Horizon Europe and beyond in the field of PM. The foreseen outcome analysis would also feed into other activities of the partnership.

3.1.4 Establishing an "EP PerMed Accelerator" for absorption of PM by users and healthcare systems

R&I projects funded under the partnership and that received funding through the ERA-Net ERA PerMed will be required to consider the potential for technology transfer in their projects from the outset. An active portfolio management approach and innovation acceleration measures will be developed (for example through a network of technology transfer offices, TTOs) to support

[8] Horizon 2020 scale for TRL: https://ec.europa.eu/research/participants/data/ref/h2020/other/wp/2016_2017/annexes/h2020-wp1617-annex-g-trl_en.pdf

the translation of novel insights into innovative applications and to ensure that these applications will reach the market and the end users.

The EP PerMed Accelerator will also ensure the incubation of newly identified projects until market readiness and will help to attract public and private funding. The EP PerMed Accelerator may also reserve budgets for patenting findings from EP PerMed funded projects.

EP PerMed will develop mechanisms to fund such projects (e.g. through RITC calls), depending on the Technology Readiness Levels (TRL). Appropriate funding mechanisms will be solicited. Typically, these projects should start at TRL four or five. The aim of these funding activities is to bring the projects by the end of the funding period at least one TRL level higher and ensure adoption in a user environment.

The EP PerMed Accelerator will also introduce reversed technology transfer initiatives in which end-users will be invited to flag the needs and desires of the broad user communities, including professionals and citizens. Researchers from academia and industry will be invited to address these needs.

3.1.5. Contribute to the development of supporting frameworks, policies and activities necessary for implementing PM approaches

To achieve the operational objective 4, the European partnership will support the development of policies, guidelines and standards as necessary supportive frameworks for PM development and implementation. For instance, EP PerMed will assess and evaluate strategies to provide evidence of good practices and standards for data sharing or clinical approaches. The partnership will facilitate the development of international standards as support for R&I, for better policy decisions and sharing of information about existing infrastructures, platforms, databases and cohorts, to avoid duplication of efforts in policy development.

PM is based on large data sets including diverse medical data collected around the individuals. In close collaboration with the respective stakeholders, the partnership will develop recommendations, for example on minimal datasets, and foster the implementation on common clinical practice regarding data collection and sharing in participating countries. By connecting already existing databases and accelerating the harmonisation of newly generated data, the value for PM will increase. This will be done in synergy with other initiatives that have similar and complementary goals, such as EJP/EP Rare diseases, IHI-JU and EP THCS.
Scientific grass-roots activities will be supported to drive the standardisation of data, processes and models in the PM field. For a long-term sustainability, the development of standards and their publication via European (CEN/CENELEC) and international (ISO) technical committees will be supported. These standards aim to provide recommendations and requirements for processes and data in PM.

Common evidence and shared information are crucial for aligned policies across Europe. The EP PerMed good practice catalogue is likely to provide major contributions for policy development aligned across Europe along the value chain, showcasing tested solutions whether for data management, clinical interpretation tools, healthcare system organisation, reimbursement models, or other aspects relevant for PM adoption in healthcare.

Another base for policy development is the international network and promotion of synergies among stakeholders, which will allow the integration of multidisciplinary perspectives supporting complex policy decisions regarding PM implementation. Finally, the strategic documents produced by EP PerMed can further stimulate policy decisions that are aligned

between regions and countries as well as globally. Differences in the maturity level between the national/regional healthcare systems will thereby be recognised and can inform appropriate action. The development of an agreement on common strategic documents is essential for an alignment on a common PM vision and future objectives, e.g. towards a permanent organisation and coordination of PM activities, e.g. through a European Institute for Personalised Medicine including representations of all types of stakeholders or other transnational collaboration in healthcare, for the benefit of patients and citizens and the society as a whole.

3.1.6. Dissemination, knowledge exchange, communication and patient empowerment actions

The activities to achieve the operational objective 5: Education, knowledge exchange and creating awareness, are described in this section. With dedicated dissemination and communication activities, knowledge exchange as well as actions toward patient empowerment, the partnership will work towards a shared language and understanding of PM for citizens, patients, and health professionals. EP PerMed will have leading role together with the participating organisations in the successful implementation of PM in a global context.

A main focus of the partnership is to provide a platform to inform and empower citizens and patients regarding PM. EP PerMed will support the development of literacy programmes, including ethical, regulatory and data-control aspects, so that patients and citizens are able to participate in decisions related to their personal healthcare. Moreover, the partnership will ensure that the perspective of patients and citizens is central in policy development and in research strategies. PM related training activities for representatives of patients, affected families, care givers, and citizens in general will be developed and implemented by the partnership. Trained patient representatives could act as advisors in research funding/evaluation processes and other R&I and implementation activities along the full value chain.

EP PerMed will further promote capacity building and the education of all health professionals to facilitate the adoption of PM approaches in clinical practice. This will involve collecting better evidence on the gaps of knowledge and needs for training the diverse health professionals (e.g. medical doctors, nurses, pharmacists, therapists, data analysts and others), with a strong emphasis on digital literacy and interpretation of biomarker information. Based on such evidence, EP PerMed can promote discussions and provide recommendations for *curricula* and life-long education of medical and other health professional, as well as raise awareness regarding the value of multidisciplinary teams for adoption of PM approaches in clinical practice. This can for example be supported by dedicated communication materials for end-users being developed, delivered and widely distributed by the partnership.

Capacity building activities regarding the benefits of PM for citizens and healthcare systems will equally target healthcare managers and policy makers, promoting informed decisions when addressing effectiveness, efficiency, equity, and ethical issues underlying the development and implementation of PM approaches.

3.1.7. Management and coordination of the partnership

EP PerMed will guarantee appropriate governance, programme management and facilitate the information flow and cooperation within the partnership, with other related initiatives and the diverse stakeholders. The activities described above will be coordinated and managed by EP PerMed through dedicated pillars and governance bodies. The governance is further described below in section 3.3.

3.2 Resources

The European partnerships for Personalised Medicine will build on the work of ERA PerMed, ICPerMed and the ICPerMed related CSAs. ERA PerMed and ICPerMed together constitute an international network of ministries and PM funders and thereby an engaged pool of members as well as access to a broad public and private PM research community in basic, translational, clinical but also on implementation, economic and social research (e.g. on patient involvement, education and training, ELSA topics) ensuring the success of the EP PerMed calls. Furthermore, ICPerMed established a partnering tool for researchers and a mapping database for funding opportunities which the partnership will build on. Multiple strategic publications of ICPerMed, such as the Action Plan and the Vision Paper, and of the ICPerMed related CSAs are fruitful sources for the development of the SRIA, the strategic joint transnational call (JTC) development and to consider and deploy the potential of regional involvement. Last but not least, ICPerMed and its related CSAs have established a multitude of contacts and cooperation, especially for the internationalisation of PM activities. These networks will be shared, merged and maintained in the partnership to increase collaboration and to fulfil the objectives. The partnership will only be successful if all members and associated partners are and remain committed from the preparatory phase (e.g. PM SRIA process in 2022, proposal preparation and submission) via the official start until the end of the envisaged duration of the EC funding and beyond. To achieve the defined objectives and impact, binding commitment to their contributions for the transnational research consortia and other important activities will be necessary. Members will be either research and innovation programme owners / managers or suitable and eligible healthcare systems as well as PM related entities. In addition to the EC budget, in-kind contributions such as event hosting or support to carrying out defined activities are also envisaged. Traditionally, all EP PerMed members participating in joint transnational call activities allocate only annual commitment for funds (predominantly through national and regional funding). The partnership will aim at long term funding commitments of the members and check whether other approaches could be suitable and feasible.

Members (beneficiaries, see also section 3.3.1) of the partnership are requested to support the proposal stage, incl. the PM SRIA process and development in 2022, and finally sign the Grant Agreement (GA) and to work actively towards achieving the overall aims and impact of EP PerMed. They commit to the EP PerMed activities (e.g. financial support provided to joint funding activities) with a dedicated budget, are eligible to be beneficiaries and receive EC reimbursements, e.g. in form of travel and accommodation to join meetings or of defined staff efforts. Clear budget commitments are expected from the funding organisations participating in the joint funding activities. According to a survey launched by ERA PerMed within the consortium and the ICPerMed family, the majority of participating funding organisations plan for increased funding and in-kind commitments, or at a minimum, stable investments in PM in the future.

For EP PerMed **associated partners** (see also section 3.3.2), not signing the GA, there is no monetary commitment required to join the partnership. In return, no financial support is provided for their participation, their contribution will be in-kind. Associated partners commit by signing a letter of intent to working actively towards achieving the overall aims of EP PerMed.

It is not expected that all members and associated partners of the partnership will participate in all pillars, but combination of different types of activities will be possible. Integrating new members or associated partners in the course of the EP PerMed is possible while the overall structure will not be impacted. The partnership will seek participation of all European countries, several of its regions and international organisations.

Based on the ERA PerMed and ICPerMed experiences, emphasised through internal surveys for

the majority of organisations it is important to receive financial support for their participation in the partnership, e.g. reimbursement for management in form of personnel costs and for travel incurring connected to internal meetings. The role and commitment (budget and time invested) of each members/beneficiary in the EP PerMed has to be clearly defined at the beginning of the partnership. Organisations/beneficiaries of EU Member states and associated countries are eligible to receive EC contribution for research funding in form of top-up when participating in a call co-funded by the EC. International partners and non-beneficiaries, including associated partners, that cannot receive EC contribution, will also be encouraged to join as funders of calls to be launched within EP PerMed.

Members and associated partners will agree before the start of the partnership on a common PM strategic and innovation agenda (PM SRIA, expected 2022) helping to align European, regional and national strategic agendas in the field of PM. The expected impact of joint funding activities for the regional/national research communities is high and includes the establishment or maintenance of transnational collaboration also with non-European countries, the promotion of research dedicated to PM and support of innovative research.

EP PerMed aims to be a global leader in PM by joint transnational coordination and funding efforts.

The total budget of the partnership is currently estimated to be a minimum of 334 Mio € for research funding and other transversal and implementation costs. This amount includes 30% of EU contribution for a seven-year programme. Thereby the investment of both EC and Member states and associated countries will be boosted when compared with previous efforts in ERA-NET Joint Calls. The currently active ERA PerMed did on average fund transnational research consortia with over 25 M€ per call and only one co-funded round. With more participating funding organisations and an expected higher commitment, an even greater total budget for EP PerMed is expected. At least one joint transnational call (JTC) will be issued per year for the first 7 years of the partnership. Additionally, in-kind contribution of the majority of the members are encouraged, e.g. as hosts for events. It is envisaged to have the duration of the EP PerMed grant set as 10 years, of which the first 7 years will be devoted to most of the activities, including calls for proposals, and the last 3 years reserved for allowing the final projects to finish. Other operations will be scaled back in this last phase of the grant.

After a preliminary assessment, around 20 M€ have been estimated to be necessary for the whole duration of the partnership for coordination and management as well as costs for the completion of all activities planned.

To take on board regulatory needs and achievement supporting the successful research and transfer of PM into the health systems, communication, networking and cooperation as well as specific tools are planned with national competent authorities (NCAs) and the European Medicine Agency (EMA). Similarly, activities for the societal and policy uptake of the results of the funded transnational consortia and the EP PerMed strategic, supportive and network activities and documents is foreseen. The partnership aims to broaden investments within and beyond the partnership research and implementation. The planned personalised medicine strategic research and innovation agenda (PM SRIA) will be a crucial instrument for this. This document will not only be the major blueprint for the research calls of EP PerMed, but also used for further activities in communication, cooperation and dissemination.

3.3 EP PerMed Governance

The governance of EP PerMed includes both a strategy/decision-making, and operational level (see also fig. 4). The different bodies are outlined below while the concrete functioning, the main tasks and working processes in the partnership, the role of the EP PerMed members, associated partners, the European Commission, the Advisory Board and the stakeholder group as well as related initiatives, etc. will be described in a Terms of Reference document.

With its dedicated member and associated partner organisations, EP PerMed will provide a platform to initiate and support PM research, to communicate and exchange on funding and PM implementation, to align strategies, educational activities as well as policy development and dissemination. EP PerMed aims at involving organisations from all European countries and from international partners. This will allow for alignment of research and funding activities at the European and international level.

3.3.1 EP PerMed – Members (GA signatories)

The **EP PerMed members** (beneficiaries) are composed of European and international (associated countries to Horizon Europe) stakeholders such as Health and Research Ministries, regional authorities as well as funding agencies and foundations in the areas of R&I and technology. The EC will be a member of the partnership. Members are requested to sign the Grant Agreement (GA) and to work actively towards achieving the overall aims of the partnership. They commit to the EP PerMed activities (e.g. financial support provided to joint funding) with a dedicated budget and are eligible to receive reimbursements, e.g. in form of travel/accommodation for joining meetings or of personnel costs.

3.3.2 EP PerMed – Associated partners

Further public and private 'not-for-profit' health research funding, policy organisations, payers and healthcare institutions can join the partnership as associated partners (i.e. associated partners in the context of the GA). For **EP PerMed associated partners**, there is no monetary commitment required to join the EP PerMed. They do not sign the GA. In return, no financial support is provided for their participation. Nonetheless, by signing a letter of intent associated partners commit to work actively towards achieving the overall aims of the EP PerMed consortium. They will be expected to report annually on their activities and to actively participate in the running of the initiative. Associated partners will have the possibility to participate in internal meetings of the partnership, contribute to and integrate their perspectives on PM into the discussions and activities of the different pillars and actively foster and drive PM developments.

Inclusion of new members and associated partners during the course of the partnership will be possible, bearing in mind that the overall budgetary envelope of EP PerMed will remain unchanged. The consortium will seek participation of all European countries and international organisations from different continents.

3.3.3 EP PerMed – Stakeholders

Organisations and initiatives with a strong interest in PM, but not able to participate as a member or associated partner in the partnership, can join the partnership activities through the **stakeholder group**. This could include e.g. payers, research institutions, universities, health institutions, patient initiatives/organisations, platforms and infrastructures, related projects or industry organisations, bodies with a public service remit at local, regional, national or international level or civil society organisations including foundations and non-governmental organisations (NGO).

3.3.4 EP PerMed – Advisory Board

An **Advisory Board** of around 10 senior experts with overall PM knowledge and collective expertise throughout the value chain will support keeping partnership activities coherent with the SRIA.

Different *ad hoc* expert groups will be formed depending on the task to be tackled related to the EP PerMed work programme. Those boards will consist of experts covering the scope/area of PM topic(s) needed for the respective exercise/work to be performed.

Experts participating in such advisory boards will have an **observer status** within the partnership. They do not sign the GA, nor provide a letter of intent. They do not represent a certain organisation but are solicited for their specific expert view. Observers are not involved in decision making processes within the partnership.

3.3.5 EP PerMed – Governance Bodies

The EP PerMed is organised around an **Executive Committee** consisting of all partnership members and partners (fig. 4). The Executive Committee is supported by a coordination body comprising an elected chair and two vice-chairs, the pillar/work package leads, the EP PerMed Secretariat and the European Commission. The coordination body is a sub-group, taking care of the consortium's everyday work.

The Executive Committee elects the **partnership chair and two vice-chairs** for two years each. Their responsibility is to chair Executive Committee meetings, to lead the coordination body and to represent the consortium externally. The chair and the two vice-chairs can be re-elected once.

The **EP PerMed Secretariat** consists of 3-4 organisations supporting the coordination body and the Executive Committee in logistics (financial and administrative) and organisational aspects. The **Secretariat coordinator is also the coordinator of the partnership** and the official contact point towards the European Commission.

At the operational level, the activities of the partnership are implemented through dedicated pillars. The different pillars comprise EP PerMed members and associated partners and are led by EP PerMed members. This aspect is essential to ensure the feasibility of the proposed work and the commitment of members taking on the respective task. Associated partners are invited to participate in the different pillars and work packages without receiving financial support. Each pillar can be subdivided in different work packages with dedicated leads. The pillars are interconnected but shall work independently and autonomously if the work requires, for example, a certain level of confidentiality, as it is the case in joint funding activities.

As the main decision taking body, the Executive Committee is central to the EP PerMed structure. EP PerMed members, associated partners and observers nominate representatives to be part of the Executive Committee. Several organisations from one country can join the Executive Committee, while in the case of decisions each country has only one vote. This applies to both EP PerMed members and associated partners. Each country will identify the entity that is acting as spokesperson. The EC, as a member, will participate in decision and voting processes.

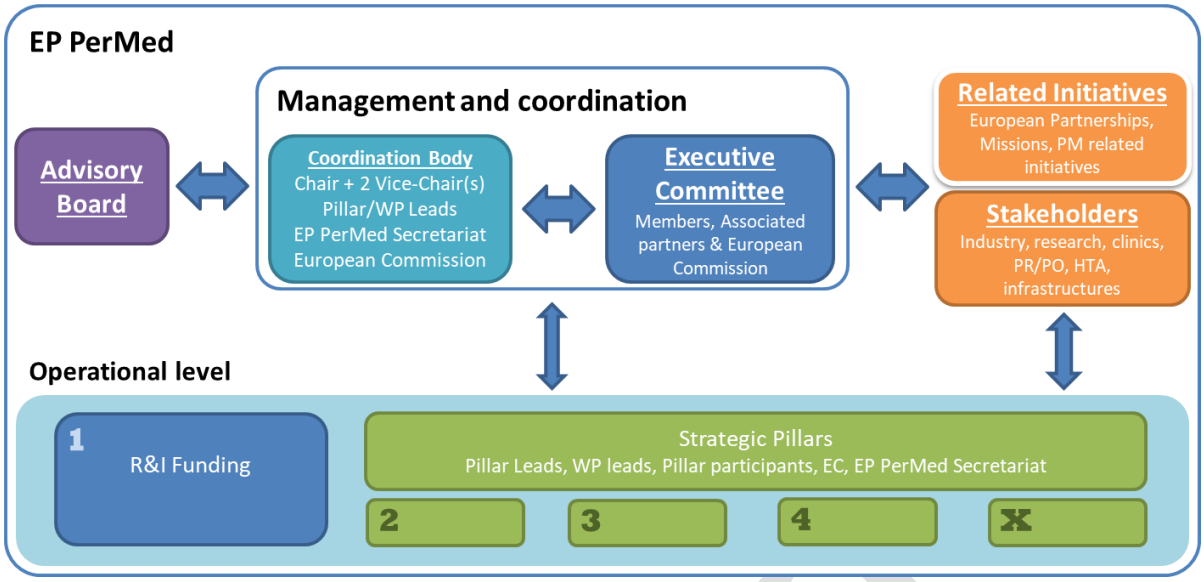

Figure 4: The EP PerMed governance structure

3.3.6 EP PerMed related initiatives

To ensure coherence and synergies within the research and innovation landscape, to advance successfully in PM implementation according to the SRIA, and to complement to the work proposed with other health topics, EP PerMed will create synergies with related initiatives. This will be in particular the following foreseen European Partnerships: "Transforming Health and Care Systems", "Health Innovation", "One Health/AMR", "Rare Diseases", ERA4Health" and "Pandemic Preparedness", Institutionalised Partnerships as "Innovative Health Initiative (IHI)" and other Health Programmes as the "Global Alliance for Genomics and Health (GA4GH)", "1-Million Genome Initiative", EU4Health as well as the Missions in Horizon Europe, particularly the Mission on cancer as part of Beating Cancer Action Plan.

3.3.7 The role of the European Commission in the EP PerMed

EP PerMed will be developed and implemented jointly with the European Commission and particularly with the DG RTD, DG CNECT and DG SANTE. The EC will be a member of the partnership and be represented on the relevant governance bodies. The commitment of the partnership towards global priorities, specifically EU objectives as the Green Deal, the Digital Agenda and Resilience, will be considered as appropriate and highlighted in the impact pathway submitted in the partnership proposal. Through this framework, EP PerMed objectives will be closely linked with these overall goals.

3.4 EP PerMed - Openness and Transparency

EP PerMed will maximise its impact by involving all relevant organisations in its structure as members, associated partners and stakeholders, aiming to broaden participation beyond current core partners. Furthermore, the partnership will be open for new collaborations and the integration of new organisations during its lifetime and across different activities. EP PerMed will foster openness and transparency on three different levels:

1. Creating synergies, dedicated communication and dissemination of the EP PerMed;
2. Open science in funded research projects (joint funding activities);
3. Recommendations around openness and transparency development by EP PerMed.

EP PerMed will develop different means of communication and dissemination strategies tailored to different audiences among the PM key stakeholders and players. Furthermore, it will implement and maintain an information channel through a dedicated partnership website and social media, enabling information to be shared about work plans, outputs such as protocols, data, results, and also strategic developments and other outcomes.

EP PerMed plans to develop an open access publication strategy for the partnership itself including recommendations around openness and transparency for the PM community, as well as for results obtained during the joint funding activities that will have to follow the FAIR[9] principles. Sharing of strategic documents and outcomes of the partnership are key success factors to achieve PM implementation and acceptance of PM approaches. Transparency and communication are thus needed for successfully translating research into clinical practice but also for improved education and literacy of citizens, patients and healthcare providers, e.g. for a better understanding of diagnostic and treatment options and towards future prevention. Furthermore, dedicated communication with higher authorities is needed to allow and enable a future uptake of PM in regional and national healthcare systems. With the inclusion of all European countries and several international partners as well as the high-level participation of ministries, EP PerMed will foster cross-border collaborations with the goal that citizens and patients will benefit from the most suitable diagnostic and treatment option available regardless of their home region/country. A high level of openness and transparency regarding a common vision will be achieved by EP PerMed thanks to the involvement of international organisations and stakeholders from different sectors. Other possibilities to inform and engage stakeholders will be explored, if applicable and feasible, in due time.

Creating synergies is one essential objective of the partnership including consultations of the PM community around the entire value chain and allowing new organisations to enter, participate in and benefit from its activities, and add value to the partnership. As there are different levels of participation, involvement of external stakeholders is possible anytime, will be actively pursued and highly welcome without compromising the ownership and commitment of EP PerMed member organisations.

EP PerMed seeks the involvement of all EU Members States and regions and international partners, independent of in the maturity of PM within their R&I or healthcare systems, as well as involvement of PM related initiatives and others via the stakeholder group to integrate their perspective but also to include all relevant key players that could benefit from the partnership's outcome.

Considering, the long funding duration and a steadily advancing PM environment, EP PerMed will act in a dynamic way, adjust its focus areas over the time span of the partnership and take advantage of emerging opportunities during the lifetime of the partnership. By following well-defined annual work plans while simultaneously being responsive and allowing a level of flexibility, EP PerMed will adapt approaches to the key bottlenecks for PM implementation as they evolve.

[9] http://ec.europa.eu/research/participants/data/ref/h2020/grants_manual/hi/oa_pilot/h2020-hi-oa-data-mgt_en.pdf

4 Annex

4.1 List of abbreviations and their individual explanation

1+MG project	One Million Genomes Initiative
AMR	Antimicrobial resistance
CAB	Call Advisory Boards
CSA	Coordination and support action
DG CNECT	Directorate-General for Communications Networks, Content and Technology
DG RTD	Directorate-General for Research and Innovation
DG SANTE	Directorate-General for Health and Food Safety
DNSH	Do Not Significant Harm
E&T	Education and Training
EATRIS	European infrastructure for translational medicine
EC	European Commission
ECRIN	European Clinical Research Infrastructure Network
EJP	European Joint Programme
ELSA	Ethical, Legal, and Social Aspects
ELSI	Ethical, Legal, and Social Implications
EMA	European Medicine Agency
EP	European Partnership
EP PerMed	European Partnership for Personalised Medicine
EP THCS	EP on Transforming Healthcare Systems
ERA PerMed	ERA-Net Cofund Action on personalised medicine
ERA-Net	European Research Area Network
EU	European Union
EU-Africa PerMed	Building Links Between Europe and Africa in Personalised Medicine
EULAC PerMed	Widening EU-CELAC policy and research cooperation in personalised medicine
ExCom	Executive Committee
FAIR	Findable, accessible, interoperable and reusable
GA	Grant Agreement
GA4GH	Global Alliance for Genomics and Health
HEcoPerMed	Healthcare- and pharma-economics in support of the International Consortium for Personalised Medicine – ICPerMed
HERA	European Health Emergency preparedness and Response Authority
HTA	Health technology assessment
IC2PerMed	Integrating China in the International Consortium for Personalised Medicine
ICPerMed	International Consortium of Personalised Medicine
IHI-JU	Innovative Health Initiative Joint Undertaking
IMI2 JU	Innovative Medicines Initiative Joint Undertaking
IT	Information technology
JPIs	Joint Programme Initiative
JTC	Joint Transnational Call
NCA	National competent authorities
NGO	Non-governmental organisation
PERMIT	Personalised Medicine Trials
PM	Personalised Medicine

PO	Patient organisation
PR	Patient representative
R&I	Research and Innovation
RD	Research Development
Regions4PerMed	Interregional coordination for a fast and deep uptake of personalised health
RITC	Research Innovation and Technology Calls
SAPHIRe	Securing the Adoption of Personalised Health in Regions
SINO PerMed	Widening Sino-EU Policy and Research Cooperation in Personalised Medicine
SRIA	Strategic Research and Innovation Agenda
TRL	Technology Readiness Levels
TTO	Technology transfer office