ICPerMed
INTERNATIONAL CONSORTIUM

Workshop Report

4th ICPerMed Workshop

Preparing the Future for Personalised Medicine: EP PerMed

17-18 January 2023
Pamplona, Spain

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Content

I- Executive Summary

The International Consortium for Personalised Medicine (ICPerMed) is a platform of over 30 European and international partners representing ministries, funding agencies, and the European Commission (EC). ICPerMed aims to provide a flexible framework for cooperation between its member organisations. The central objective of ICPerMed is to align and encourage joint efforts in personalised medicine (PM) research and implementation.

This is the report of the 4th ICPerMed Workshop, entitled "Preparing the Future for Personalised Medicine: EP PerMed", which took place in Pamplona (Navarra, Spain) on January 17-18, 2023.

The workshop was held in a hybrid format to ensure the participation of all interested and invited parties. The workshop assembled almost 80 international experts in all areas of PM in physical presence and about 40 experts participating virtually.

The workshop was developed within the objective of the ICPerMed Secretariat Work Package 2 "Stimulating cooperation and transfer of knowledge", with the support of all members of the Secretariat. The ICPerMed Secretariat committed to the organization and support events in order to establish a forum for the discussion and development of research and strategic priorities in the field of PM, as well as the development, the sharing and the dissemination of standards, best practices, the needs and barriers to implement PM and guidelines. Moreover, the ICPerMed Work Package 4, leaded by the National Health Institute Carlos III (ISCIII), on "Accelerating translation, implementation and innovation" provided input in the content of this workshop including a session focused on the "Best Practice of Overcoming Personalised Medicine Implementation Barriers". In addition, the ICPerMed Executive Committee, EC representatives, invited international experts, partners of topic-related European, national and regional projects as well as ICPerMed Advisory Board members contributed to an important and lively discussion.

The workshop aimed to continue formulating and improving the personalised medicine Strategic Research and Innovation Agenda (SRIA) of the European Partnership for Personalised Medicine (EP PerMed), especially identifying some key areas, such as innovation system, implementation system, involvement of industry, health care systems, education, patient representatives etc. EP PerMed will accelerate transnational PM research, its impact for innovation and its implementation into the health systems for the benefit of each individual. Many international institutions are working together in preparing this proposal. EP PerMed will begin at the end of 2023.

The workshop facilitated the discussion and the exchange of experiences, ideas, perspectives between the ICPerMed members and international and regional high-level experts in the field. It was organised around four panel sessions focused on four key areas in PM: Patient and citizen engagement and education; International and regional perspectives; Innovation and industrial collaboration; and Health system implementation. One plenary session followed by a panel discussion related to best practices in PM field and the identification of needs, gaps and barriers in the implementation of research projects as well as their overcome. A plenary lecture around a success example of PM implementation in the local healthcare system of Navarra. In addition, there were one plenary session around the introduction to EP PerMed, and other focused on the presentation of EP PerMed SRIA.

Panellists and international and national experts in the field of PM discussed the main topics proposed, which successfully will contribute to redefine EP PerMed SRIA and the development of the future proposal. The active and fruitful participation of panellists and experts, both physical and virtually, will allow to collect and take into account more, new and crucial insights into the challenges involved in implementing PM

approaches. Therefore, this workshop has also allowed updating the SRIA and gathering key essential information and developments for the foreseen annual joint transnational calls, new direction of activities, events, approaches, appropriate tools and instruments, that will be developed in the coming years in the field of PM.

As results of the discussions, there was agreement on a high necessity to increase efforts, and to prioritize the next steps in the following areas, focused on these objectives:

- Educational efforts: Improving patient and citizen engagement and awareness together with the enhance of the skills of the PM workforce are key points to ensure the PM implementation in a fair and equitable way.
- Building trust is the basis for convincing patients. To communicate the value and evidence of PM, such as prevention, diagnosis and treatment.
- Make efforts in training all the stakeholders, researchers, innovators, healthcare providers, healthcare specialists, patient, citizens.
- There is much inequality in the implementation of PM among the different countries of the world. Political, economic, legal and socio-cultural factors make it impossible to develop PM projects in many regions.
- Building trust among public and private partnerships, as well as building more synergies and communication between all actors involved in PM. It is essential to connect the industry, large pharmaceuticals companies, the academia, services providers and other national and international entities that collaborate in research and development.
- Legal and ethical regulatory framework in order to develop better systems to generate data, to store data, to access to data, to share and reuse data.

The outcomes of the 4th ICPerMed workshop are published by ICPerMed and will be integrated into the SRIA and EP PerMed, as well as into future recommendations, guidelines and other strategic publications.

II- Workshop Introduction

The fourth ICPerMed workshop entitled "Preparing the Future for Personalised Medicine: EP PerMed", was held at the Baluarte Congress Centre and Auditorium of Pamplona (Navarra, Spain), on the 17th and 18th of January 2023. The National Health Institute Carlos III and the Government of Navarra, from Spain, hosted the Workshop with the financial support of the European Commission.

The Workshop was structured in the following plenary sessions, divided in two days.

On Tuesday 17th January:

- Open plenary session.
- The ICPerMed "Best Practice in PM" Recognition 2021-2022 Award ceremony.
- Plenary session of introduction to EP PerMed and EP PerMed Strategic Research and Innovation Agenda (SRIA) and draft proposal presentation.
- First Panel: Patient and citizen engagement and education.
- Plenary session and Panel discussion focused on "Best practice of overcoming PM implementation barriers".
- Second Panel: International and regional perspectives of PM.

On Wednesday 18th January:

- A keynote lecture "A success example of PM implementation in the health care system of Navarra".
- Third Panel: Innovation and industrial collaboration.
- Forth Panel: Health system providers, payers, insurances and medical societies.
- A plenary session of closing remarks.

III- Welcome & Introduction

Cristina Nieto, from The Spanish National Health Institute Charles III (ISCIII), also the coordinator of ERA PerMed and member of the ICPerMerd Sec, gave a welcoming speech and introduced the ICPerMed consortium to all the present and online attendees. She also thanked to the European Commission to provide the opportunity to organise this workshop and to the Government of Navarra for hosting this event.

Cristina Nieto introduced the topics of the workshop, which are the different key aspects of the SRIA in PM and EP PerMed that needed further contribution for its development: Patient and citizen engagement; International and regional perspectives; Innovation and industry collaboration; and the role of Health systems in PM. She underlined the importance that all these topics move towards to accelerate the implementation of PM in the healthcare systems.

Due to currently, several national and regional institutions are working together in the preparation of the proposal of the new European Partnership for PM and the SRIA, Cristina Nieto encouraged the active participation of the attendees to contribute with their expertise, their knowledge and new perspectives and insights in order to contribute the SRIA and proposal of EP PerMed.

Then, Cristina Nieto introduces the speakers of this Welcome & Introduction session.

The first one was **Mikel Irujo**, as hosted from the Government of Navarra. Mikel Irujo is a PhD in European Law. Since February 2021, he is appointed as Regional Minister for Economic and Business Development of the Government of Navarra.

He began his opening speech by thanking ICPerMed for the initiative to hold the Workshop in Pamplona and warmly welcomed all the attendees for coming to Pamplona. He underlined that PM is a strategic priority in Navarra. Numerous business willingness and talent have emerged in this region in recent years. The region of Navarra offers a very favourable ecosystem for the promotion of bio- entrepreneurship that is being progressively consolidated. Navarra has an important concentrate knowledge centres in the biotechnological area, infrastructures, health services, the administrative integration, as well as the highly skills and specialization industrial sectors, with more than 80 companies. Moreover, Navarra has two renowned universities with a long academic tradition, reputable public and private healthcare network closely link to the universities as well as centres research (Navarrabiomed, IdiSNA, CIMA), with around 31.000 people in the care system, 2.500 people working in health industry and more than 1.000 researchers, a region with around 650.000 habitants. Navarra also has the 17% of patents in the pharmaceutical and biotechnological area compared to the 30% of patents in Spain. The Government of Navarra is strongly committed to the development and consolidation of the biomedical sector in the region. This has led to a very positive increase in the region's economic growth over the last five years.

Their strategy for PM was born in 2020 and it supports the industry, as well as provide specific funding programmes for technology, development and innovation in PM. Mikel Irujo mentioned one example, as One Thousand Genomes Project, called NAGEN. This project is headed by Ángel Alonso from Navarrabiomed. The NAGEN Program is a strategic initiative to implement genome analysis in the clinical practice of the Navarre Health Service.

For all these reasons, Navarra wishes to commit and contribute to the new SRIA of PM and collaborate with the European initiatives.

After that, the elected Chair of ICPerMed **Ejner Moltzen** intervened. Ejner also gave a welcoming speech to the attendees and underlined the importance of this Workshop to provide inputs to the SRIA, which is a required to start the new European Partnership in PM. What we have been working on in recent years and more specifically in this moment in the preparation of the SRIA, is that preclinical research is already advancing in the implementation of PM. He also focused that in the field of PM there are many challenges and it is important to identify what kind of needs and issues must to be solve and to find new solutions and to provide in this new SRIA the directions and guidelines to go forward and truly support the future of PM. Ejner also highlighted the fruitful role that the Navarra region has played in recent years in the field of PM, being a regional benchmark for the achievements that have been reached.

The European Commission representative, **Carmen Laplaza Santos** (Head of Unit for Health Innovations & Ecosystems) introduced the European Commission's perspective on PM. The European Commission congratulated ICPerMed for its work, because the implementation of PM is already a reality in the clinical practices.

European Commission has been supporting PM over the last decade, investing more than 2 billion euros in collaborative research projects to cover different angles, instruments and actions in PM.

PM in Horizon Europe is in Cluster 1 (Health, Research and Innovation), considered a crucial pillar. One of the main motors of Horizon Europe is the impact of research and innovations.

What does European Commission want to achieve with the future cofound projects in PM?

- The translation of the research and innovation for the benefit of citizens, patients, healthcare professionals and healthcare systems. In this line, EC has seen how some of the projects it has co-financed have become a reality, but it is still committed to continue making efforts to ensure that the projects are replicable, to translate scientific evidence into clinical practice, such as Pharmanagen in the pharmacogenomics field, developed by Navarrabiomed and awardee by ICPerMed Best Practice Recognition in 2021.
- Another critical point in the co-found projects by the European Commission is to ensure that all the actors (research, healthcare professionals, regulators, payers) are well represented.
- Another important player to make real the implementation of PM is regions. How can regions contribute to the implementation of PM in clinical practice? In many cases, regions are responsible for the research implementation; they are also responsible for the healthcare provisions, also for the education, and for supporting and pushing the industry tissue. She also underlined that the affordability, access and availability of PM are key point for European Commission, in order to all European citizens have the same health opportunities to receive personalized medicine. In the future, the healthcare someone receives will not depend on where people live.

To finalise, Carmen Laplaza concluded her introduction by mentioning that many European political initiatives really need PM approaches, such as Europe´s Beating Cancer Plan and Cancer Mission. Different European Commission programmes support these political ambitions, for example under the Digital Europe Programme, two project truly close to PM have recently started, by the end of December 2022. The European Genomic Data Infrastructure (GDI) that aims to implement the objectives of 1+ Million Genomes Initiative, in order to enable secure access to human genomic, phenotypic, and clinical data across Europe. In addition, the European Cancer Imaging Initiative, whose aim is to foster innovation and development of digital technologies in cancer treatment and care, to achieve more precise and faster clinical decisions, diagnostics, treatments and predictive medicine for patients who suffered cancer.

IV- Plenary Session: Introduction to EP PerMed

Wolfgang Ballensiefen, as EP PerMed Chair introduced to the audience the EP PerMed background and the development process of the proposal, with the timeline.

The European Partnership for Personalised Medicine (EP PerMed) is one of the European research and innovation partnerships under Horizon Europe, specifically to Horizon Europe Cluster 1 – Health, which will be dedicated to improve health outcomes within sustainable healthcare systems through research, and the development and implementation of PM approaches for the benefit of patients, citizens and society.

This effort is not starting from scratch as existing initiatives provide a strong basis for establishing a successful EP PerMed. It is built on the International Consortium for Personalised Medicine (ICPerMed), the ERA PerMed and the so called "ICPerMed Family", which are several Coordination and Support Actions funded by the European Commission, that support the research and implementation of PM in Europe and beyond. Four CSAs dedicated to the communication and cooperation with Latin American and Caribbean countries (EULAC PerMed), China (Sino-EU PerMed and IC2PerMed) and Africa (EU-Africa PerMed). Two CSAs focused on European regions (Regions4PerMed and SAPHIRe); another two CSAs with research topics (HEcoPerMed and PERMIT) and one CSA on personalised prevention (PROPHET).

He also explained gradually the EP PerMed preparing activities, highlighting some critical actions and dates in the process and development of the proposal. For example, the first step was to design the Preparatory Group of ICPerMed, ERA PerMed and the European Commission in 2020-2021. Another important point in this process was the starting of a European Commission, Member States Group and a Drafting sub-Group in September 2021, as well as the starting of the proposal Writing Group with EP PerMed partners in November 2022. He also pointed two main future dates, such as the EP PerMed proposal submission, that will be on April 13th, 2023. Moreover, EP PerMed will start after a positive European Commission/HADEA review and a grant agreement signed (around Q4/2023).

Currently, a EP PerMed Draft Proposal, developed by the Drafting Group has been published by the European Commission, as well ICPerMed and ERA PerMed, in February 2022.

He also continued his speech, explained that the EP PerMed EC call of is open: HORIZON-HLTH-2023-CARE-08-01. The expected outcomes from the EC call are detailed below:

- European countries and regions, along with international partners, are engaged in enhanced collaborative research efforts for the development of innovative PM approaches regarding prevention, diagnosis and treatment.
- Healthcare authorities, policymakers and other stakeholders develop evidence-based strategies and policies for the uptake of PM in national or regional healthcare systems.
- Health industries, policymakers and other stakeholders have access to efficient measures and investments to allow swift transfer of research and innovation into market.
- Health industries and other stakeholders can accelerate the uptake of PM through the adoption of innovative business models.
- Healthcare authorities, policymakers and other stakeholders use improved knowledge and understanding of the health and costs benefits of PM to optimise healthcare and make healthcare systems more sustainable.
- Healthcare providers and professionals improve health outcomes, prevent diseases and maintain population health through the implementation of PM.

- Stronger and highly connected local/regional ecosystems of stakeholders, including innovators, are in place and facilitate the uptake of successful innovations in personalised medicine, thus improving healthcare outcomes and strengthening European competitiveness.
- Citizens, patients and healthcare professionals have a better knowledge of PM and are better involved in its implementation.
- Stakeholders cooperate better and establish a network of national and regional knowledge hubs for PM.

The European Partnership for Personalised Medicine will be implemented through a joint programme of activities ranging from research to coordination & networking activities, including training, demonstration, piloting and dissemination activities, to be structured along the following main building blocks:

- Joint implementation of the SRIA.
- Joint annual calls for R&I activities, applied R&I, pilots.
- Capacity building activities.
- Activities to enhance the skills of the relevant personalised medicine workforce, and improve citizen relevant awareness and literacy.
- Deployment activities through pilots, innovation procurement and financial support mechanisms.
- Flanking measures.

He also introduced the expected budget for EP PerMed, as well as some examples of activities developed by the Writing Group in supporting the proposal.

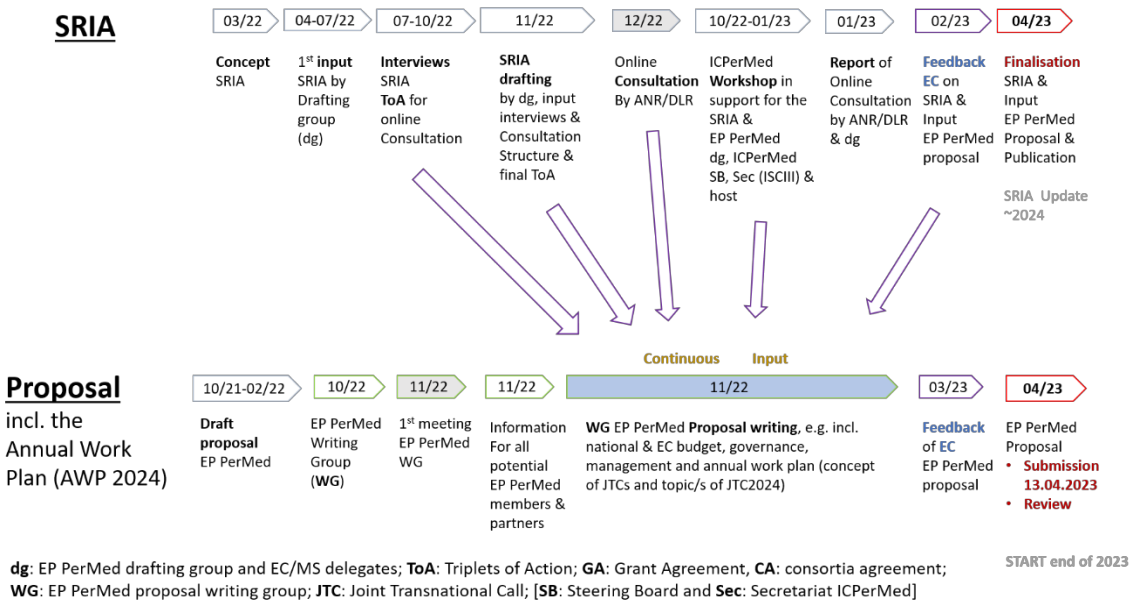
- Member States, including Regions, and the EC will invest over 300M€ support transnational calls, other activities of EP PerMed and its management for 7-10 years.
- Minimum 7 co-funded PM-related Joint Transnational Calls, comparable to the 5 implemented by ERA PerMed (2018-2022), will be developed, executed and monitored.
- Additionally, networking and pilot calls are planned to support the development of innovative PM approaches and their sustainable implementation into health systems.
- The partnership aims be the major communication and event platform for PM in cooperation with ICPerMed other initiatives and the EC.
- EP PerMed will communicate and align its activities with the other European Partnerships of the Health Cluster and related initiatives/projects in Europe and beyond.

Finally, Wolfgang pointed that this ICPerMed workshop aimed to collect crucial input for EP PerMed and its Strategic Research and Innovation Agenda (SRIA), through the key note lectures and the 4 different plenary panel discussion dedicated to the following PM related topics (Patients and citizen engagement and education; International and regional perspectives of PM; the PM innovation system and industrial collaboration; and PM implementation into the health systems).

V- Plenary Session: EP PerMed Strategic Research and Innovation Agenda (SRIA) and Draft Proposal Presentation

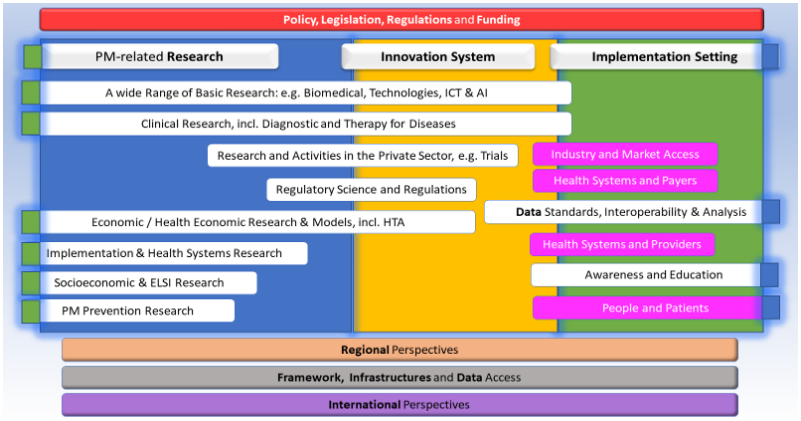
Monika Frenzel, as EP PerMed co-Chair introduced to the audience an overview and an update of the EP PerMed Strategic Research and Innovation Agenda (SRIA), which is currently in preparation.

The European Partnership for Personalised Medicine (EP PerMed) proposal, which it is also in preparation, will be directly connected to the SRIA. The parallel process of the development of the SRIA and the EP PerMed proposal is shown schematically below.



The SRIA will guide the EP PerMed activities, such as funding (Joint Transnational Calls) and other activities as trainings, events, etc.

The EP PerMed SRIA will identify gaps, needs and challenges as well as opportunities and visions, which should be addressed, overcome or reached to maximise the benefits of PM approaches, within sustainable health systems and to ensure that there is an equal access to these approaches. For this purpose, the SRIA will address the following topics:



- Interdisciplinary research (basic, translational and clinical) in all related areas.
- Innovation system.
- The implementation setting.
- International and regional perspectives.
- Framework, infrastructures and data access.

For the development of the SRIA, the consortium together with international experts and stakeholders have collected inputs, information, ideas and analysed reports for EP PerMed and its SRIA. For this purpose, the drafting group of the SRIA, with kind support of the European Commission, organised around 70 interviews with international experts in the field and stakeholders. The interviews were translated into the key elements **Triplets of Action** (ToA) related to personalised medicine. A triple of action consist on a sequence of challenge/objective/outcome, that allows to identify specify gaps, needs and actions in a structured and comparable way. So far, over the different areas and chapters of the SRIA a total of 47 ToA were identified.

In addition, ICPerMed launched of an online consultation (21 November – 31 December 2022) for the SRIA in support for the EP PerMed, for the following purposes: 1) Communication of the SRIA in general and the identified ToA in specific; 2) To obtain general input, especially related to the ToA; 3) Input regarding the urgency and timing of the ToA; and 4) To obtain suggestions for additional ToA.

According to the structure of the Consultation, the Triplets of Action were presented in 4 categories:

- Personalised Medicine related Research (ToA numbers 1.1 – 1.17).
- Innovation System and Personalised Medicine (ToA numbers 2.2 – 2.8).
- Personalised Medicine Implementation Setting (ToA numbers 3.1 – 3.16).
- Overarching Activities (ToA numbers 4.1 – 4.6).

Experts in the field of PM contributed to the SRIA Consultation following this way:

- Validation and urgency of the presented ToAs.
- Selection of 5 most important ToAs per section.
- Selection of 5 most important ToAs considering all 47 ToAs.
- How 15M€ would be distributed amongst the selected 5 ToAs.
- Possibility to propose new or redefine presented ToAs.

Monika Frenzel also presented the first outcomes obtained in the SRIA Consultation. A total of 714 interested viewers; 129 surveys submitted and analysed and 21 countries participated in the SRIA Consultation.

Based on their expertise, the participants have identified crucial ToAs from the 4 different categories, as well as they provided 10 different topics to include as ToAs and suggested 13 ToAs adaptations. Related with research, 72% of participants selected the ToA “A collaborative approach between pre-clinical and clinical research” as the most important aspect, followed by the ToA “New targets for personalised therapies making use of an improved understanding of disease mechanisms”. Seventy-five percentage of participants identified the ToA entitled “Early cooperation between public research and private sector” the most urgent topic to develop in the SRIA related to the Innovation System, followed by the ToA “Expanded knowledge on valued for personalised medicine”. According to the Implementation Setting, around 50% of interviewers pointed two ToAs “Training and Education of healthcare professionals” and “Test Beds in hospitals”. Finally, around 80% of participants have also reported two ToA in the field of Overarching Activities. The first one, “Network of national and regional innovation hubs” and the second entitled “Connected large-scale health databases”.

She showed the first preliminary results of the SRIA Consultation. A deep analysis is ongoing developing, and it will be publish in the ICPerMed webpage. To finalise, Monika Frenzel, as EP PerMed co-Chair encouraged to all workshop participants to intervene and to have an active participation due to this workshop was the last input for the SRIA development before its finalization by the Drafting Group.

VI- Plenary Sessions: Panels

i. Patient and Citizen Engagement and Education

- Moderation: **Maria Jose Ruiz Alvarez**, Italian Ministry of Health.
- Panellists:
  - **Marius Geanta**, Centre for Innovation in Medicine – Romania.
  - **Larisa Aragon Castro**, National Data Stream Consortia – Switzerland.
  - **Nathalie Lambrechts**, Biomedical Research at V – Flanders.
  - **Merel Hennink**, FT3 – The Netherlands.

Maria Jose Ruiz Alvarez, as moderator of this panel discussion, introduced each of the panellists and the topics that were discussed in this session. The key items were mainly focused on the awareness and empowerment of patients and citizens in the field of PM, the transforming if the patient's role through data management, and patient and public engagement with healthcare services.

She also provided to the audience two main definitions: PM and patient engagement.

***PM** is: a medical model using the characterization of individuals' phenotypes and genotypes (e.g. molecular profiling, medical imaging, lifestyle data) for tailoring the right therapeutic strategy for the right person at the right time, and/or to determine the predisposition to disease and/or to deliver timely and targeted prevention.*

***Patients Engagement**: involving patients as active participants and central stakeholders in implementation Research in PM. Simply enrolling and following patients as passive research subjects in a clinical trial does not rise to the level of patient engagement. Instead, patients should be treated "as valued and valuable partners whose input, advice, and guidance are sought and implemented" throughout these processes. Also, families, carers and users of the health services should be involved in the whole process.*

The panellists also contributed with their perspectives and gave their own definition of patient engagement: For Merel Hennink, patient engagement is involving patients in research as equal partners. For Nathalie Lambrechts, it is giving the patients the control of their own data and an easy access to those data for different applications. Marius Geanta also intervened saying that patients are often perceived as mere passive subjects receiving care. Patients engagement is first not considering only patients but also all the citizens that are not patients as active part of the healthcare systems. Setting the proper models at governance level for involving citizens as well as patients in the healthcare system is the key of engagement.

After that, the moderator asked some questions to the panellists. All of them intervened, as well as the audience also contributed to the discussion.

1. **How can patients effectively participate in Personalised Medicine throughout the development pipeline, adding value to the process and achieving the full potential of Personalised Medicine, with a focus on areas such as clinical trials design and the use of diagnostic devices?**
   Patients have the power to influence the future of healthcare. It is necessary to involve more patients in the prioritization of the research questions, to involve patients and citizens in the study design and to provide support in all the stages of the projects, as well as the valuation of the patients and citizen's involvement impact on research. For example, Cancer Mission is a good example of prioritization of the involvement of patients and citizens as they were included from the first step of research and their input has been taken into consideration to prioritize choices.

Patients have experience in supporting larger groups composed of different stakeholders. They also have experience in specific diseases and caregivers and PAOs (Patient advocacy organizations) are of great support for technical knowledge and regulatory aspects. Thus, it would be necessary to clearly know what the researchers want for patients, how exactly engage patients each in their own way and who does what.

It is important to design a framework enabling patients to contribute to the information provided to research. In real life there is a connection between socio-economic aspects and science: the socio-economic status of patients influences the biological response. Therefore, social sciences should be included in research and used to predict factors that can affect health, genetics, etc. Moreover, another key point is how to spread the information; it could be useful to build a model to disseminate the researched results and implementation in lay language.

2. **Patients' role has been transformed. Are there indicators and instruments that can be used to evaluate patient engagement in Personalised Medicine?**

   For example, in countries like The Netherlands, patients are really engaged in research and in the writing phase of the research proposal and they also have a budget for their participation and clear expected outcome. Having clear expected outcomes makes easier to create decision models from outcome made by patients and to create and evaluate the patients' impacts. These very good patient engagement initiatives can be used as example to follow by others. In addition, in Italy it is asked to the researchers where the patients, as well as stakeholders, can contribute in the research and care. From this work, 115 indicators of engagement were set for patient and stakeholder. What is important in the setting of research is to prioritise what the research needs from patient and stakeholders. In this way is possible to create a model to engage patient and stakeholders.

3. **How to ensure awareness and empowerment for all citizens, not only patients? How citizens can improve their knowledge of health/disease under the PM approach? How can the partnership make the patients aware of their involvement?**

   A fundamental point to the engagement is the spreading the information through the whole society to make people confident about the opportunities of PM, but also the challenges. Mainly clinical care professionals can inform patients, but the whole society must be informed. Each individual should be looked at from different perspectives and adapt the way in which the information is transmitted to the citizens. One message does not fit all. Therefore, different kind of messages need to be provided in order to make citizens aware of the innovations and to make them trust medical doctors and devices, politicians, healthcare systems, etc. The inequality gap among the aware and not aware people must be filled with universities, hospitals, local level information and dialogue. The obligations to spread the information and to educate people is shared by all the stakeholders, but mainly by Ministries of health and funding institutions. Local authorities can influence and engage in a more personalised way, for example, general doctors and the primary care is fundamental to transmit awareness. Patients may not want to be involved, but it is important to emphasize the responsibility of patients, which it is closely linked to value-based healthcare.

   Value-based healthcare is a healthcare delivery model in which providers, including hospitals and physicians, are paid based on patient health outcomes. Under value-based care agreements, providers are rewarded for helping patients improve their health, reduce the effects and incidence of chronic disease, and live healthier lives in an evidence-based way.

4. **What is needed to enhance the "pull effects" for Personalised Medicine by patients, people and the healthcare system players in addition to the "push effect" by research and policy?**
   - Patients join from an individual perspective and a social dimension. Patients and citizens should be an active partner. Currently, there are some initiatives already in place. For example E-body project provides a tool in which patient choose a clinical trial and give impressions. Moreover, in the PFMD project, it is explained how to engage patients in research.
   - European Commission and Governments should create a virtual educational programme for physicians to facilitate the creation the model involving patients in a social dimension.

5. **What would be the priority for the implementation of PM from the point of view of patients, in one year from now?**
   - New treatments.
   - The availability of data infrastructures, reliable and open to patients – legislation – shared decision-making models.
   - Inclusion of social sciences and humanities.
   - Discussion going not into how to engage patients but on how to ensure diversity and inclusion in patient engagement.
   - Availability and access to the genomic data needed for particular and general clinical care.

6. **What advice for the future partnership for implementing patient engagement?**
   EP PerMed could be the tool to standardize across Europe the involvement of patients and citizens, to design guidelines or structure to be used in every country.

The Investments delivered so far in the different initiatives focused on PM have been productive and now we start seeing the results. However, the continuous fostering of research and implementation and the effective participation of patients in the entire PM pathway are still crucial for realizing the full potential of PM. Improving citizen awareness together with the enhance of the skills of the Personalised Medicine workforce are key points to ensure the Personalised Medicine implementation in a fair and equitable.

ii. International and Regional Perspectives of Personalised Medicine

- Moderation: **Monika Frenzel**, ANR.
- Panellists:
  - **Gianni D' Errico**, Tuscany Life Sciences – Italy
  - **Maria Jose Ruiz Alvarez**, Italian Ministry of Health – Italy.
  - **Étienne Richer**, IRSC |CIHR Institute of Genetics – Canada.
  - **Joaquín Guinea**, Innovatec – Spain
  - **Ángel Alonso Sánchez**, Navarrabiomed – Spain

Monika Frenzel, as moderator of this panel discussion, introduced each of the panellists and the topics that were discussed in this session. The ideal to be achieved is that the services provided by health systems should be independent of the place where we live, thus it is necessary to better integrate transnational collaboration. After that, the moderator asked some questions to the panellists. All of them intervened, as well as the audience also contributed to the discussion.

1. **What is the status of PM activities in research and on the implementation level and which are the main achievements of Personalised Medicine in the country/region and the main challenges you face?**

   A region is an ideal location for PM pilot projects. To date, at the national level, research is not only funded by big funding agencies, but also by regional entities. In recent years, very good research groups have emerged in regions that were previously not funded, and this has been thanks to initiatives such as ERA PerMed, ICPerMed or Regions4PerMed, which has made it possible to establish connections between different national and transnational regions. There is an increasing willingness to go for change in many regions, greater willingness to cooperate; even many regions have updated their research and innovation strategic agendas. Increasingly, different and diverse regions are betting on their inclusion in research projects, demonstrating that PM is possible and worthwhile. It is now a reality that there are many examples of good practice throughout Europe. This is due to the efforts of European Commission, the will of regions to modernize, the breaking down of barriers and being willing to change and invest in PM. These new updates highlighted the same challenges, although the implementation pathway is different because the regions are different.

   A remarkable feature of international collaboration projects is that biomedical and clinical aspects are not only the aspects that have to be included. At international level, the key point is how to integrate the ethical, legal, regulation, social aspects, as well as the economy. The implementation of PM in different health systems varies internationally, and clinical and genetic algorithms may even have to be reformulated so that they can be applied to that other population in that other country. All kind of international communities should be included in clinical studies, both researchers from respective countries as well as populations. However, other regions do not have the same possibilities and funding sources as the first world countries. It is in our hands to give the possibility to contribute to PM in those regions with limited resources, and to facilitate the cross boarder health.

   One remarkable example are Latin American (LA) countries, which show a very heterogeneous landscape of PM implementation. Some of them, such as Brazil, Argentina and Uruguay are showing a lot of interest in PM, developing high numbers of projects with a very high level of preclinical research, also they are working in PM regulation. By contrast, other Latin American countries cannot do that. LA countries show willingness in the possibility of cooperating in international PM projects. Some countries, such as Argentina, for example, have some gene analysis initiatives in pathologies such as cancer and chronic diseases. Other challenge in LA countries is the technology, with has different regulation and application in health systems.

   Another example is PM in Africa, in which there are big differences between countries in PM implementation. The case of South Africa is different from the other African countries. Overall the inclusion of diverse populations specifically the African populations not only assist Africa but can lead to new market opportunities in therapeutic and diagnostics industries making this regional understanding a critical juncture for the global PM agenda.

2. **What kind of activities could be interesting to be performed by EP PerMed to foster PM research, development, innovation and implementation in international, transnational and regional collaboration?**

   Data sharing and storing would be one of the main challenges. Data sharing is difficult because common framework is missing. Panellist suggested that EP PerMed could contribute to the

development of new frameworks building on existing ones, with transnational regulation. Uniform tools and standards would be a great value within and beyond Europe. Also, to promote FAIR principles.

To promote and facilitate transnational cross-border initiatives, as well as regional investment, based on regional guidelines and support from local authorities.

Regarding LAC countries, EULAC PerMed project has already finished. EP PerMed could try to maintain the cooperation with LAC countries, to maintain the scientific community created or to try to create networking groups, as well as to invite these partners to be part of EP PerMed. In addition, other initiative could be to have networking grants or starting grants, which involve LAC and African countries with European countries, despite it is an extra effort. In fact, some LAC funders joined ERA PerMed Joint Transnational Calls.

The governments from countries under development often do not see the potential value of PM, or the real economic impact of PM. Maybe it is better to have focus on improved general health of society than on health economic perspectives in our communications. Due to the networking groups have already created, the next step may be to promote interactive transnational training, to facilitate a real exchange.

At international level, the challenge is to be a global leader in PM. Some activities could be developed to achieve this goal, such as organization JTC, mapping of non-EU PM initiatives, organization of international workshops and conferences, training and capacity building activities, as well as promotion and coordination of common PM standards.

From the point of view of regions, it is important to leverage the outcomes and networks created by regional CSAs. In the innovation side, regions have different kind of involvement in PM. To facilitate the translation of preclinical research into the clinical practice is necessary the support of local government and even in most cases there are technical agencies who manage research innovation infrastructures that also support it. EP PerMed should try to connect the different regional parties already created and with experience (research institutions, entities, researchers, funders, infrastructures…) to move forward to the implementation of PM.

With the aim of facilitating the exchange and the interaction between different regions in order to replicate best practices examples, a possible idea could be the use of twinning instruments.

Other key issue highlighted is how to convince regions, how to set the priority to PM. What is the cost of no implement PM approaches? We have the evidences of cost-efficiency of PM approaches. At this point, call of responsibilities, convince policy makers with strong evidence, to present and invite authorities to get to know regional best practices examples, also to consider good communication strategies and levering the right communications tools, such as local press. Maybe, it is necessary to communicate what are the economic and health consequences of not applying PM.

iii. Innovation System and Industrial Collaboration

- Moderation: **Ejner Moltzen**, Chair of ICPerMed, Innovation Fund Denmark.
- Panellists:
  - **Jean-Marc Bourez**, EIT Health – France.
  - **Nathalie Seigneuret**, IHI Europe – Belgium.
  - **Niklas Blomberg**, ELIXIR – UK.
  - **Natasa Debeljak**, University of Ljubljana – Slovenia.
  - **Jens Habermann**, BBMRI-ERIC – France.
  - **Maria Orr**, AstraZeneca – UK.
  - **Andrew Thomson**, EMA – The Netherlands.

Ejner Moltzen, as moderator of this panel discussion, introduced each of the panellists, as well as the topics that were discussed in this session.

1. **What are the challenges to overcome "valley of death" for PM related research and innovation?**
   The core challenge is to identify precision medicine approaches that make a difference for patients. To do this we need to drive clinical transformation, for example, promoting preclinical to clinical translation, in order to facilitate and speed the bench to bedside transition. Today's research is tomorrow's healthcare.
   In order to drive fundamental research and development and to deliver more evidence for biomarkers for precision medicine, other challenges mentioned were to act fast on the data problems, like the access to connected large-scales health databases, access to high quality medical cohorts with associated biomarker and molecular data, and access to biobanks and real-world data registries.
   According to research and clinical aspects, some issues were mentioned: more and better biomarkers, the improvement of clinical strategies, the validation of different patient cohorts, the implementation of PM in other diseases not only oncology and show the cost-effectiveness of PM approaches.
   Other key aspects noted to drive acceleration on research and innovation were to foster public-private-partnerships also on regional/local level, as well as engaging patients, patient representatives.
   In terms of financing, the panelists highlighted the need to reduce the administrative burden and allow for the maintenance and implementation of results (e.g., longer project duration). Funding flexibility that would make it easier 1) for industry and academia partners to join projects; 2) to address ad hoc needs (time delay between call preparation – project conduction – upcoming clinical needs) and 3) to enable local/regional public-private partnerships. More simplified administrative processes in data processing, taking into account data protection.
   Related to training and education, some aspects have been mentioned, i.e. the need for multidisciplinary and continuous training due to the rapid progress in biotechnological applications, the development of high-tech devices and the management of big data. In addition, it is necessary a good basic natural science education for researchers, health professionals, and to bring the natural sciences closer to patients and citizens.

2. **What are the main areas for R&I investments in PM approaches today and tomorrow?**

   A highest priority topic mentioned by several panelists is the creation of easily accessible, federated, comprehensive, well-curated, connected and widely accessible large-scale health databases and bio-repositories, as well as biobanks, which are very powerful tools, but further investment is required to maximize information mining.

   Take more attention on education and training in order to improve the understanding and power of precision medicine and drive uptake with clinical professionals and also patients.

   Technically, substantial investments are in genomics while PM can only succeed to the fullest based on the most comprehensive view. Hence, technological advancements for PM are important in the following domains, such as any other Omics technology (Proteomics and Metabolomics), infrastructures, systems medicine and artificial intelligence and patient empowerment tools.

   Currently, PM in oncology with Molecular Tumour Boards can be perceived as role model and seems to dominate main areas for research an innovation investments. This experience from the implementation of PM in oncology should be expanded towards other pathologies, such as infectious and inflammatory diseases, paediatrics and geriatrics medicine, rare diseases, among others.

   Also, more interdisciplinary approaches, going towards “One Health”.

3. **What are the highest priorities in relation to innovation and industrial collaboration in PM?**

   One of the major obstacles is the fragmentation of initiatives/funding within and across the different levels (regional, national, EU, beyond EU) as well as the unawareness of already existing tools, expertise and good-practice examples; in short, using more efficiently synergies cost- and resource-efficiently should be a major focus.

   Lower administrative and regulatory burden; both also being closely linked to the fragmentation challenges.

   The SRIA might leave us medicine regulators be perceived as a hurdle put in front rather than using us as an integral part of the personalised medicine process and as one of its enablers (through interactions and integrated advice that we offer; covering HTA, ITF, scientific advice on relevant topics from biomarker qualification to trial design and authorisation, labels; ACT-EU on multi-stakeholder topics etc.)

   The SRIA would benefit from using the need to advance regulatory science for personalised medicines as a driver for stakeholders, academic and commercial, in their products to also generate results or by-products (transferable solutions) that can be used by regulators to more efficiently recommend the next personalised medicine. Regulatory science research in this area can range from compelling principles for discovery and non-clinical data models, to simple but clever design tweaks to clinical trials that deliver extra information at no extra cost to developers, to methodologies for clinical prediction models that inform different questions of stakeholders, from health professionals discussing with patients to medicine regulators to HTA.

4. **What kind of support is needed from the involved stakeholders and players?**

   In terms of research, panelists pointed the improvement of clinical trial strategies, to speed up de the development and validation of diagnosis and biomarkers, as well as more funding. Areas like regulation, clinical care and market access need to be carefully revised to fit PM approaches. Moreover, approvals could be accelerated, but it must be clear which patients can have benefits for which drug. Also, the role of regions. A lot can be learned regarding building trust between stakeholders from regions due to the closeness of stakeholders.

5. **Is there a need for public-private partnerships in the future - and why?**
   Panellists argued that trust between public and private players is key, but this has developed and improved over the years. Not in all cases, PPs are the best model: it must be considered carefully from case to case whether a PPP is interesting and relevant.
   The true value in PPPs is the collaboration itself, not the monetary side of it.
   For example, IMI has provided many valuable PPPs projects with valuable outcomes, but more is needed.

6. **What are the most important aspects to include in the EP PerMed SRIA?**
   Panellists mentioned some key aspects that they considered crucial to include in the SRIA of PM. New reimbursement or payment models are needed, timely access to patients in need, considering the balance between high price expectations of the industry and the long-term sustainability of health care systems. This challenge is jointly faced by industry parties, health care providers/payers and patients alike. The way to address properly this challenge could be addressed throughout the value-based healthcare approaches, meaning that patient registries become central to support this strategy. In case, failure to address these challenges may result in a weaker market position for European Research and Industry, in favour of the US and/or Asia, and that citizens in Europe are not provided the best affordable possible treatment options.
   Artificial Intelligence (AI) in precision medicine. There is a challenge on reproducibility and bias in the use of AI methods. This will increasingly be brought to the fore with the new AI act. It would be desirable address both larger, diverse datasets (leveraging the national partners) as well as independent validation in new cohorts / settings (again using the diversity of national partners).
   Other important point mentioned is about the importance to think about innovation and industry participation in every layer of PM. In this context, for example, ELIXIR have run a series of industry workshops and it is not only about pharmaceuticals and novel treatments. There are opportunities for innovative companies that cater to local markets across Europe (e.g. in Spain there are large investments in local language AI text models that cater to healthcare).
   In addition, funding and support of mini research projects in Personalized Medicine, approaching a specific challenge with a multidisciplinary team of students and co-mentors, with a background in several disciplines, including medicine, genetics, biotechnology, computer science, electrical engineering, economics, law, etc. This mini project could support research at MSc level (similar to current student projects within European Social found) or PhD program.

iv. Personalised Medicine Implementation into Health Systems

- Moderation: **Hemma Bauer**, Australian Ministry of Education, Science and Research.
- Panellists:
  - **Astrid Vicente**, Portuguese Health Institute Doutor Ricardo Jorge – Portugal.
  - **Toni Andreu**, EATRIS– The Netherlands
  - **Paula García**, ECRIN – France.
  - **Ramiro Gilardino**, Global Health Technology Assessment Policy Lead – Switzerland.
  - **Terje Peetso**, North Estonia Medical Centre – Estonia.
  - **Juan José Beloqui**, PharmaNAGEN Coordinator, Navarrabiomed – Spain.
  - **Stefania Boccia**, Cattolica Del Sacro Cuore University – Italy.

Hemma Bauer, as moderator of this panel discussion, introduced each of the panellists, as well as the background and the topics that were discussed in this session.

Over the past years, there have been nice examples of implementation of PM approaches into the health care systems. ICPerMed has collected and communicated about best practice examples via its website. But still, implementation approach into health care systems entails a lot of challenges.

The first one is that the way how health care systems are set up is to serve providing health care to populations but are not focussing on individuals. PM is kind of a disruptive model to the existing health care systems. Moreover, organisational wise there are many challenges: PM approaches are usually complex, requiring multidisciplinary collaboration. It is still lacking in place an adequate culture and structures in health care that facilitate this multidisciplinary collaboration. PM approaches might require new technologies that are not used or are not available in the routine health care. Moreover, PM requires new infrastructures, for instance data infrastructures to provide access to data for PM diagnostics and treatment. It is also due to PM often go with additional investments and costs that contradict the sustainability of health care systems, and the regulatory framework in place (GDPR, etc.) might not be the most supportive to implement PM approaches.

Therefore, the aim of this panel session was to get input what type of EP PerMed activities should take up in the SRIA in order to address these issues and to support the implementation of PM approaches into health care. Important to mention is that EP PerMed will not have the mandate itself to implement PM approaches, this stays under the sovereignty and responsibility of the national and regional health care system and health care providers. Nevertheless, PM may set activities in order to support and to facilitate the implementation into health care.

The questions discussed in this panel were the following:

1. **What crucial aspects need to be considered or anticipated by research initiatives developing novel PM approaches to promote implementation in health care?**
    - Discovery research is not the only need: confirmative validation research studies are highly needed as well. Research needs to present strong scientific evidence: there is the need to focus on quality of research and on techniques and methods used; otherwise research lacks reproducibility of results and scientific evidence. Confirmatory research should be a multistakeholder process.
    - The knowledge on the necessary regulatory processes along the value chain needs to be increased. There is still a lack of knowledge on regulatory requirements among the research community. Strong effort on training is needed. Also, training of people to access and use the data and prevention is necessary.
    - PM approaches require data infrastructure in health care. However, data management and data governance also require investment. That is a challenge for the health care system, therefore support needed to build up these infrastructures.
    - Patient empowerment is also needed. This also goes along with training measures for patients, patient advocates, health literacy, health care providers, HTA, etc.
    - Apart from the widespread communication on PM and PM approaches is needed to increase dialogue between multidisciplinary stakeholders; patients, providers, payers, researchers and also communication and dialogue with politicians is highly needed.
    - Prioritization on research strategic plans between regions, because regions cannot follow all.

- PM is pushing for changes in healthcare systems also via needed multidisciplinary teams.

2. **When listening to public health experts, the topic of prevention is very high on the political agenda. However, when you consider the reality of healthcare, prevention is not as prominent. What are in your opinion, the main challenges and opportunities for personalised prevention?**
   First, panellists gave the definition of personalised prevention.
   Personalised prevention aims to prevent onset, progression and recurrence of disease and/or through the adoption of targeted interventions that consider the biological information, environmental and behavioural characteristics, socio-economic and cultural context of individuals. This should be timely, effective and equitable in order to maintain the best possible balance in lifetime health trajectory.

   It is important to communicate on the increase of quality of life and the increase of life expectancy. It is necessary to create the evidence that by the personal prevention measures you create value and that you are promoting health within society. Biology, social and economic aspects and behaviour are the pillars of health.
   Once the evidence is proved, results should flow into guidelines in order to widely implement the PM prevention measures. Subsequently, implementation research is needed to monitor and evaluate the measures and the implementation.
   Concerning prevention new technologies come into place. Facilitators for personalised prevention come from novel technological developments that allow collection of very detailed real world data - EHR, wearable devices, omics technologies. This will allow both research for prevention and profiling of people for personalised prevention.
   In addition, push to improve the primary health care systems. The former primary and secondary prevention categories fall together in the single term PM prevention.
   Also, it is necessary to look for mechanisms to take into account and to assist patients outside the hospitals.
   All panellists were in the same line: prevention is a critical point, taking into account that citizens will live longer, but they also aim to live better as well, without the burden of diseases.
   Panellists also pointed the value-based healthcare aspect. The focus should be on the outcome of the measures. It is necessary to demonstrate that the investment is worthwhile, to convince healthcare providers of this, even if it entails additional costs at the beginning. But what is invested today is secured for tomorrow.
   It would be convenient to add new approaches for public health and to develop new cost models to foster PM that bring together the different stakeholders and converge at the same point: what is important to patients and what is important to the healthcare system?

3. **What main measures and activities should be considered by the EP PerMed SRIA in order to promote and support the implementation of PM approaches into healthcare systems?**
   - To create strong evidence on research outcomes: confirmative validation studies are needed. Also, research on evaluation of health care measures that are already implemented.
   - Support measures to make data FAIR (Findable, Accessible, Interoperable and Reusable), i.e. develop guidelines, and develop new instruments and tools.
   - Foster multidisciplinary processes and platforms.
   - Training, in particular to patients, payers, health care providers. To disseminate the value of PM between general society. Also, change the mind: PM has to be seen as a tool for all disciplines. PM should be the way of doing things.

- Foster regulatory research.
- A sustained dialogue between stakeholders: payers, patients, researchers, health care providers, innovators, infrastructures, industry etc, in order to define research priorities together.
- Foster on public-private partnerships (PPP).
- Foster on value-based healthcare.
- Team up with the European Health Data Space (EHDS).
- Convince policy makers to implement research into the health care systems.
- Investments into ELSI research.
- Data access and data sharing. Development of tools that make data available, analysable and shareable should be undertaken.

VII- Plenary Session: Best Practice of Overcoming Personalised Medicine Implementation Barriers

The goal of this session is to show successful examples of PM projects and the main barriers that they encountered during the implementation of their research projects and how they tried to overcome these barriers. For this purpose, this session counted on the participation and experience of two coordinators of projects funded by ERA PerMed.

Alberto Ortiz, co-funded ERA PerMed Project 2018 Call "Multidimensional stratification for treatment of acute kidney injury – Kidney Attack Project", from the Health Research Institute of Jiménez Díaz Foundation, Madrid, Spain.

Violeta Serra, funded by ERA PerMed Joint Transnational Call 2019 "Patient stratification based on DNA repair functionality for cancer precision medicine – RAD51 Predict Project", from the Vall d' Hebron Institute of Oncology, Barcelona, Spain.

First, Dr **Alberto Ortiz** presented a brief summary of his research project and then explained the barriers that his research group have found.

Acute kidney injury (AKI) kills 2 million people every day. In AKI, kidney function fails suddenly and may later recover spontaneously. The diagnosis is later, and there is no treatment for AKI that accelerates the recovery and patients may require replacement of renal function by dialysis. Kidney Attack aimed to change this status quo by innovative and smart combination of scientific literature, preclinical and clinical studies that explore multiple markets (i.e. systems biology, omics) to propose early personalised detection of AKI and novel treatments. Earlier diagnosis of AKI or of AKI risk will allow well-designed randomized clinical trials assessing treatment.

During the development of his research projects, Dr Alberto Ortiz and their research team have found five critical points.

- A clinical need regarding the need for early diagnosis of AKI or earlier assessment of increase risk.
- The need of industry partner that can provide the product.
- Technology available and in clinical use.
- If implementation result in improved outcomes.
- And if implementation is cost-effective.

Currently, they do not yet know whether the implementation of the results obtained in this research project will improve the outcomes of the patients and if it will be cost-effective. In order to answer these two questions , which are the main barriers found in the clinical implementation, they need: 1) to develop pragmatic clinical trials, which the main barrier is funding; 2) To decide to initiate therapy early based on u-peptidomics results, whose barrier is to reduce turn around time; and 3) To assess impact on outcomes, in which they found that select the drug is the main barrier.

Afterwards, Dr. **Violeta Serra** also described first the research project that she coordinates, and then the barriers encountered during the development of the project.

Tumour with DNA repair defects, such as those from BRCA1/BRC2-mutation carriers, respond very well to certain chemotherapies and to new targeted drugs named PARP inhibitors (PPARi). They have developed a

new test based on the visualisation of a DNA repair protein named RAD51 that enables to establish if tumour cells have DNA repair defects, beyond mutations in BRCA1/BRCA2. This projects aimed to establish the predictive value of the RAD51 test in four major cancers types (breast, ovarian, prostate and endometrial) and if the test provides functional evidence for the interpretation of genetic variants of unknown significance. They also wanted to improve the test to enable its implementation in the clinic and perform an economic evaluation to compare our test versus current selection criteria for PPARi therapeutic indications.

The main barriers that they found regarding the developing alike diagnostic tests were in various aspects, such as technical, level of evidence, economic, regulatory and social.

Regarding the technical issues, they found five challenges, for which they have identified a solution:

- Immunofluorescence technique required specific microscope settings that are not available in routing diagnostic laboratories. The solution was to centralise test in trained centers interlaboratory validation.
- Optimising the test from solid tumour to liquid biopsies was solved developing new protocols.
- Manual versus automated scoring was overcome by digital pathology.
- Histology-based assay in the era of genomics was overcome proving comparative results to genomic biomarkers.
- Problems in data storage and sharing: research versus local hospital versus international partners. The solution was to design different data storage structures.

According to level of evidence, they also encountered three challenges.

- The preclinical evidence recapitulating cancer heterogeneity (n>100).
- Demonstrating clinical evidence. The first clinical trial was achieved by the access to clinical cohorts with annotated outcome and biomarkers.
- Design of prospective clinical trials. The design is not easy. They needed to team up with cooperative groups and pharma-industry.

In the field of economic, two main challenges were found:

- High cost of biomarker validation with prospective clinical trials. They need to carry out bigger clinical trials of biomarkers validation, thus they need to teamup with pharma-industry.
- High cost of development of diagnostic test at comercial level. for that they need the license to a diagnostic company, the support from an interested pharma company, which took them a lot time.

Although they are not currently facing regulatory issues, because they have not reached that point, they find that there is a need of high-level expertise EU-IVD regulation. The solution proposed is to include experts and training in the project.

In addition, she highlithed the main social aspect, which is the economical analysis. They lack real-world clinical evidence, because at least in Spain the drug is not yet giving to the patients. for this reason they assumed that it is difficult to do the analysis, and they need to add slowly the data from our prospective clincal trial in order to be able to complete the model.

The **panel discussion**, moderated by **Cristina Nieto** (National Health Institute Carlos III), as ERA PerMed coordinator and including as panellists both ERA PerMed coordinators, Dr. Alberto Ortiz and Dr. Violeta Serra, then addressed some issues not previously raised related to the main barriers encountered in the implementation of the research projects:

- Lack of funding to cover the whole value chain.
- The time of funded research projects is normally too short. Researchers need to apply for additional funding to cover all the project.
- The design and implementation of clinical trials is becoming increasingly complicated and costly. Calls for pragmatic clinical trials could be a good initiative.
- Prospective clinical trials take more time than the financial life of the projects.
- The cost increased maybe improve clinical outcomes.
- The administrative tasks, such as the preparation of call for proposal documents are time-consuming and complex.
- It is necessary multidisciplinatory teams, with experts involved in legal, economic and ethical issues. Regulatory frameworks is very complex for researchers and need a specialised support.
- Openn Access: It is key to ensure the reuse and reproducibility of the data.
- In order to build more international research consortiums and to engage more researchers, it could be useful to provide networking calls before launching the transnational projects.
- Data sharing is a huge issue in case to start a new trasnational consortium. The interoperability among different data bases is crucial.
- Other crucial point is to maintain the balance between the academia research and the innovation, including intellectual property aspects. Researchers have a proffesional aim is to publish their results, but also the innovation and the implementation issues are important to be considered.

EP PerMed will take into account the barriers described to achieve more successful implementation of research projects in the field of PM.

VIII- Lecture: A Success Example of Personalised Medicine Implementation in the Health Care System of Navarra.

Gonzalo R. Ordoñez, Director of Personalised Medicine and Laboratories at NASERTIC, a public company of the Government of Navarra. Member of the technical committee of the Strategy of Personalised Medicine in Navarra, and member of the Navarra Health Cluster.

In his speech, Gonzalo R. Ordoñez introduced the *Integrated Personalised Medicine Strategy for Navarra: Personalising Navarra. Navarra is focusing on Personalised Medicine for your health and wellbeing.*

Navarra was the first region in Spain to develop a PM strategy, which three departments elaborate it: health, innovation and economic development. Navarra strategy aims to provide improved healthcare based on the study of the human genome, as a valuable argument for bio-health research and innovation, and as a tool for economic development, that makes it sustainable, with an environment of safe, ethical and equitable technological expansion.

In Spain, Navarra is one of the regions that has been developing a firm commitment to PM over the last years to become a leader at national level. The region has extensive experience in pilot programmes through the call for strategic projects in genomics and advanced medicine, both in basic research and healthcare, with specialised staff and state-of-the-art infrastructure.

Historical perspective on the main milestones of PM in Navarra: It began in 1994, with the introduction of advances in digital health care, such as an electronic medical record for each citizen. In 2015, the government of Navarra choose the PM as one of the areas to be promoted in the specialization strategy, whose mission was to promote the development of a new way of doing medicine centred on citizens. One of the first measures taken was the funding call for strategic R&D&I projects on genomics and advanced medicine. This results in very nice examples of collaboration between basic and clinical researchers. In 2020, small companies and bigger organizations, as well as the networking between public and private entities facilitated the implementation of its PM strategy, that it is expected to cover in a 10 year period.

The PM implementation into the Navarra healthcare system has been made possible thanks to European funding calls, and regional funds. In this context, Gonzalo highlighted the importance of regional funding programmes, which have been needed to develop their capabilities in PM. One example is the initiative to implement the genome sequencing and their analysis in the clinical practice in Navarra Health System in 2016. Since then, six different programmes have been developed: 1) The analysis of more than 1000 genomes from patients and their relatives with rare diseases and types of cancer; 2) The genome sequencing on patients whose clinical results indicated a failure in the response to specific drugs; 3) The genome analysis in patients with hypercholesterolemia; 4) Paediatrics population; 5) Breast cancer and 6) To study the genetic factors underlying infertility issues.

The strategy was organised in three phases. Phase one or the design phase: drafting, describing the strategy. Phase two or the deployment phase, in which the timeline indicators and goals were established, as well as the budget for all the action lines. The third phase or the implementation phase: proposal discussion, collection of inputs and validation. Finally, the strategy got the approval by the Government.

The strategy defines the mission, vision and value of medicine, as a key element to improve the healthcare based on the study of the human genome.

Gonzalo Ordoñez highlighted some key elements of successful strategy development and implementation:

- Institutional support.
- Adaptation of the healthcare system to the implementation of the different tools of PM.
- The generation, integration of OMICS data and clinical data.
- The appropriate financing activities for adequate development.
- The provision of infrastructures for data sequencing, storage, processing and analysis.
- Regulatory framework to ensure that the generated data can be shared safely.
- The creation of a collaborative network with public and private entities, with an effective relation.
- Effective process to engage citizens, receiving their active participation in the development of PM strategy.

There are some lines of actions and the indicators to evaluate the degree of implementation of the actions. The implementation phase began in 2020, however some lines of actions had to begin later due to the covid pandemic. For example, a line of action was to create communication materials, such as summaries, guides for professionals and citizens, templates etc. Also, the preparation of informative and educational video posters in several languages.

To achieve the line of action related to data management, they considered technical aspects and issues such as market analysis, the design and the analysis of functionality of a federated platform, with regulatory and ethical frameworks. The sequencing centre and the computing centre. Since covid pandemic, these infrastructures have been allowed to sequence tens of human whole genome and hundreds of human whole axon per day, as well as a fast and a reliable storage of genomic and clinical data.

Moreover, Navarra is one of the main regions in Spain driving the national strategy of PM, through a programme called IMPaCT (the pillar to facilitate the implementation of PM in Spain). IMPaCT is organised in three programmes: predictive medicine, data sciences and genomic medicine. Navarra is involved in these three clusters.

Gonzalo Ordoñez finished his speech saying that the process of the PM strategy has begun, and it will be probably a big and tough way to work, but a very interesting and fruitful way for the coming years. In Navarra they have been and believe they will continue to witness successful examples of sustainability and innovation around PM, driving economic development in the Navarra region.

IX- Plenary Session: ICPerMed 'Best Practice in Personalised Medicine' Recognition 2021-2022

The aim of the 5th ICPerMed Recognition was to honour outstanding clinical research projects related to data sharing in PM. Awardees had the chance to present their work during a specific plenary session for further dissemination.

Chiara Ciccarelli, as ICPerMed Secretariat member and on behalf of Gaetano Guglielmi, from the Italian Ministry of Health, introduced the award ceremony for ICPerMed "Best Practice in Personalised Medicine" Recognition 2022. The winners were:

1. Dr Mario Alberto Battaglia
2. Dr Annalisa Trama
3. Dr Julia Stingl

Moreover, Dr Ludmilla Thomé Domingos Chinen, awardee in the previous ICPerMed "Best Practice in PM" Recognition 2021, had the opportunity to present her award-winning project, due to she was unable to attend the 3rd ICPerMed Workshop "Personalised Medicine: How to Ensure Value-based Implementation", that took place in Brussels (Belgium) on 21st-22nd of June 2022.

Two of four winners, Dr Mario Alberto Battaglia and Dr Annalisa Trama have been able to participate on-site in the Workshop and to present and explain their project. Dr Julia Stingl made an online presentation of her award-winning project, and Dr Ludmilla explained her research clinical project in PM through a video.

Below is a brief sketch of the awardees and a summary of their research studies.

Prof. Mario Alberto Battaglia is Full Professor of Hygiene and Public Health at the University of Siena; he is President Italian Multiple Sclerosis Foundation since 1998 and co-founder of the Italian Multiple Sclerosis Register, as well as of the Rehabilitation in Multiple Sclerosis EU Network. His research fields include chronic diseases, epidemiology, health economy, big data, health services and rehabilitation. His proposal is entitled "**Databases and registers toward Barcoding Multiple Sclerosis**" and is clustered as Example of efficient data sharing, engaging citizens, patients, health care professionals, and PM researchers, economically beneficial, and avoiding redundancies/data loss.

Multiple Sclerosis (MS) research in Italy is demonstrably "privileged" for several reasons: first, there is a well-established tradition of scientific collaboration among Italian MS Centers. They guarantee a high and homogenous level of care throughout the country. This also entails an excellent quality of work in the collection of data and samples and, above all, the ability to finalize collaborative projects. Second, resources for data collection in different domains (clinical, imaging, epidemiology, genetics, patient-reported outcomes) are already active at the national level: Italian Multiple Sclerosis and Related Disorders Register; Italian Network of NeuroImaging - INNI; PROgnostic GEnetic factors in Multiple Sclerosis (PROGEMUS); Sardinia Genomic Database; Patient-Reported Outcome Measures for MS(PromoPROMS). In line with the strategic priority of People with MS' Research Agenda promoted by the Italian Multiple Society (AISM), its Foundation (FISM) has promoted and funded the above databases and registers. The Barcoding MS project of the Italian Multiple Sclerosis Foundation of AISM is based on the idea for the creation of a code for each patient, capable of unambiguously and completely identifying his or her entire history of disease.

The COVID-19 pandemic further enhanced the above "FISM's research ecosystem"; FISM together with the Italian MS Registry, the Italian Society of Neurology and the Italian Association of Neuro-Immunology signed

the Italian Alliance on Covid-19 in MS. It allowed studying the impact of COVID-19 infection in people with MS with the immediate implementation of data collections; this made it possible to be the first in the world to provide fundamental information on the safety of immunosuppressive drugs against SARS-CoV-2 infection (MuSC-19). Also relevant here was the collaboration with industry.

Building on the excellence of the above resources, FISM has now moved a step forward, towards the possibility to integrate the different knowledge domains of these resources. This is the key action to enable research that provides unambiguous answers to fundamental questions about disease etiology, disease progression and therapy. To this aim, FISM launched the BARCODING MS Initiative that is meant to achieve the full interoperability of the existing resources, in an integrated multidimensional picture of the disease (integrate all data, create a unique disease history identifier for each individual patient), boosting not only research on disease pathophysiology and progression but also excellence in clinical care. The data collected allow doctors and researchers to better understand the effects of therapies, the evolution of the disease and to be able to shed light on the causes of MS, which are still elusive.

Dr Annalisa Trama is Director of the Evaluative Epidemiology Unit at the Fondazione IRCCS Istituto Nazionale Tumori, Milan (INT), where she also serves as Coordinator of the Institutional Strategic Research Priority on rare cancers and complexity in oncology. She is an expert in population-based research and is involved in many national and international projects exploiting secondary use of real world data (e.g. electronic health record, administrative database, clinical registries, population-based registries) and use of innovative solutions (e.g. data fusion; AI approach for defining causal relationship; AI solution to support interoperability of data sources). Her proposal is clustered as Use of innovative technologies/infrastructures for data exchange and is entitled "**The first international federated clinical registry on rare head and neck cancers"**.

Cancer care for head and neck cancers is multidisciplinary and complex and knowledge on the rare ones is limited. There is a wide consensus that to support clinical research on rare cancers, clinical registries should be developed within networks specializing in rare cancers. The hypothesis proposed by Dr Trama in this study is that the head and neck cancer registry initiated in the framework of the European reference network on rare adults solid cancers (EURACAN https://euracan.eu/) will help to: describe the natural history of rare head and neck cancers; evaluate factors that influence prognosis; assess treatment effectiveness; measure indicators of quality of care.

The registry is a prospective observational real-world registry collecting data from already available registries/database and/or directly from expert health care providers. The registry is federated (i.e. data are spread across multiple data providers). Analyses will be performed using the federated learning approach which splits computation into a local part, run independently by all data providers, and a central part. Data quality checks are envisioned to assess whether data values are present, valid and believable. Validity and plausibility checks are embedded in the electronic case report form in the form of alerts and errors during the data input. Additional checks are implemented in R. An engine, leveraging R scripts and an online instruction repository, is used to perform the additional data quality checks and to provide the Vantage6 software with the registry data it needs to perform the federated analyses. For this registry, several Vantage6 algorithms have been developed to generate descriptive statistics such as averages and cross-tabulated counts over a selected set of parties and variables. The Vantage6 infrastructure allows for a wide range of algorithms, hence algorithms at increasing levels of sophistication are under development. The data analyses will include descriptive statistics showing frequency and patterns of patients' and cancers' variables, analytical analyses investigating the association of patients/disease and/or treatment characteristics and health outcomes.

Dr Julia Stingl is Professor at the University Hospital of RWTH Aachen and head of the institute of Clinical Pharmacology. Her research is focused on the variability of the response and side effects in therapies in individual medicine, including clinical pharmacogenetics, drug metabolism, enzyme kinetics, particularly in depression and in polypharmacy in elderly patients. Her proposal is clustered as Example of efficient data sharing and is part of a project financed by ERAPerMed during the call 2021. Indeed, it is entitled **"Data sharing and re-use in the framework of the ArtiPro ERA PerMed project"**.

Depression is a major contributor to global disability. Despite intensive efforts and a large number of studies to stratify patients into discrete treatment groups, we are still far from being able to predict the individual's course of depressive illness or the outcome of treatment. The ArtiPro project (Artificial intelligence for personalised medicine in depression - analysis and harmonization of clinical research data for robust multimodal patient profiling for the prediction of therapy outcome), funded in the framework of the ERA PerMed Joint Transnational Call 2022, aims to establish a platform for artificial intelligence (AI) research that combines clinical research data on biomarker signatures and therapeutic outcomes to identify robust multimodal biomarkers and treatment outcomes for depression.

ArtiPro will take advantage of large existing cohorts, including both patients and healthy subjects with subclinical characterizations. These datasets represent a large bulk of molecular, biochemical, imaging, phenotypic and clinical data that will be harmonized taking into account their heterogeneity and complexity. This platform may allow large multimodal datasets to be used to develop predictive models for symptom domains and outcome data, thereby increasing the power of these data compared to the original individual data. The aim is to enable AI approaches to identify novel biomarker index sets that predict outcomes, providing a basis for the development of a decision support system for personalised therapy.

It is evident from the above that ArtiPro poses important ethical and legal challenges, as the project will mainly use data that have been previously collected or generated in the framework of previous projects. For this reason, in the first months of the project a step-by-step process has been undertaken to guide ArtiPro researchers in efficiently sharing existing data in accordance with legal and ethical constraints. This process, based on the careful evaluation of available international guidelines, has benefited from the advices of experts in the field of legal and regulatory research and materialized in the production of a questionnaire shared among ArtiPro partners.

A brief sketch of **Dr Ludmilla Thomé Domingos Chinen**, and a summary of her research study entitled "Higher platelet-to-lymphocyte ratio is prevalent in the presence of circulating tumour microemboli and is a potential prognostic factor for non-metastatic colon cancer" was previously published in the 3rd ICPerMed Workshop Report "Personalised Medicine: How to Ensure Value-based Implementation".

X- Plenary Session: Wrap up & Closing Remarks

Ejner Moltzen, ICPerMed Chair, **Wolfgang Ballensiefen**, EP PerMed Chair and **Cristina Nieto**, host of the workshop, formally closed the fourth workshop of ICPerMed.

They consider that the workshop objectives have been achieved and many inputs have been collected from the different plenary sessions, which will be taken into account for the SRIA. The workshop has reinforced the preparation of the SRIA, and some difficult sections such as implementation of PM have been extensively discussed throughout the workshop.

They are grateful for the active participation of the public both present and online attendees. They also appreciate the great contribution of all panellists and moderators. Special mention to the local people from the Government of Navarra for the organization of the workshop and its warm welcome.

Speakers

Panel 1: Patient and Citizen Engagement and Education	
Marius Geanta Centre for Innovation in Medicine, Romania	He is the President and Co-Founder of the Center for Innovation in Medicine, a non-governmental organisation based in Bucharest, Romania, focused on innovation in the healthcare sector. Marius Geantă is a pioneer in the field of personalised medicine in Romania and Central Eastern Europe and is involved in some pan-European innovative healthcare projects, such as Information Technology: The Future of Cancer Treatment, DigiTwins Consortium, International Consortium for Personalised Medicine, Public Health Genomics Network, PECAN, Building Blocks for Personalised Medicine. Marius Geantă has more than 15 years of experience and expertise in the field of health and science communication as editor in chief and publisher of many medical journals and health communication platforms.
Nathalie Lambrechts Biomedical Research at VITO, Belgium	She is aiming for sustainable health care by implementing technological and scientific findings from our cells to the population and society. She is involved in sustainable health research at different scientific and organisational levels: from molecular biology, cell culture to human population studies and societal impact, and from lab experience to project management. As coordinator of the We Are project in which she strives for empowerment and innovation through health data literacy for all through multidisciplinary research.
Merel Hennink FT3, The Netherlands	Merel Hennink was diagnosed with stage IV NSCLC in November 2014. Until April 2020, she worked as a program manager and a teacher at the University of Applied Science Groningen. Early 2015, she became active in the Patient Advisory Board of Longkanker Nederland (Dutch Patient Organization), to be a face and a voice of Lung cancer in the Netherlands. In 2018, she also became an ambassador of Lung cancer Europe (LuCE, an European umbrella Organization of Patient Organization) and also represents Longkanker Nederland in the Global Lung Cancer Coalition (GLCC). She had presentations on diverse platforms (ERS, ELCC, ESMO,WCLC etc.) and is active in diverse global patient councils. In 2020 she was a mentor in the IASLC STARS program. She became active in the Global Initiative of the ROS1ders in 2015. In her own country, she started the foundation Stitching Merels Wereld. As a result, the Hanze University of Applied Sciences started a research course ´Merels Wereld´ in 2017.
Larisa Aragón Castro Patient and Public Involvement Expert in the National Data Stream (NDS) Consortia, Switzerland	More than 20 years experience in various roles with a strong focus on Program and Project Management/ Cultural Transformation Change Management and Coaching of Senior Leadership Teams in the Diagnostics and Pharmaceutical worldwide leading company. Experienced in cross-functional, intercultural collaboration and customer-centric approaches. With a clear interest and drive for strategy customer benefits realization aiming to achieve tangible results, sustainable process improvements and value creation for the company and patients.

Panel 2: International and Regional Perspectives	
Gianni D`Errico Foundation Tuscany Life Sciences, Italy	He is head of Project Management Office at Toscana Life Sciences, the main regional player in the biomedical field and policy support in Tuscany Region. He is also vice chair of the International Consortium for Personalised Medicine. He obtained his degree cum laude in International Relations at Federico II University in Napoli, with a major in international economics. He obtained his Post Graduate master's degree in Project Management in Rome and a certification in Intellectual Property Management at University of Bonn (DE). He is also an EMBA laureate at Bologna Business School (University of Bologna). He started his professional experience in Brussels managing R&D projects within FP7 Programme in the Energy and Environment Sector. In 2013, he moved in Slovenia where he gained an extensive experience on the R&D project in Health and Biotech sectors for the main international players (Novartis, Sandoz, Lek, Merck, Millipore, Karlsruhe Institute of Technology, etc.). In 2015, he moved to Milan where he held the position of the European Funding Officer at Regional Foundation for Biomedical research (15 Million €/year to fund biomedical research). In July 2018 he Moved in Florence where he works as Head of Project Management Office at Toscana Life Sciences, within the Office for the valorisation of health research (UVaR) at the DG health of Tuscany Region.
Joaquín Guinea InnovaTec, Spain	Dr Joaquín Guinea is the General Manager and co-founder of Innovatec, a Spanish consultancy company with long-term expertise in R&I activities. He is also Honorary Professor of Alcala University (Madrid). He has been evaluator for R&I projects for the European Commission and for some European countries. In previous positions he has been Director of the Technology Transfer Unit of the University of Alcalá (Madrid, Spain), CEO of a biotechnology company, official Spanish representative in several European R&D Programmes. He was also appointed as chairman of the Madrid Biotechnology Industry Association for several years. He was the coordinator of the FP7 research project EVAL-HEALTH, and the H2020 project EU-Africa PerMed. He is also member of EU-LAC PerMed project.
Étienne Richer IRSC|CIHR Institute of Genetics, Canada	Associate Scientific Director in CIHR Institute of Genetics. Dr. Étienne Richer first joined McGill University as a postdoctoral scientist in genetics of infectious diseases. Dr. Richer then joined BioMedCom Consultants where he was involved in projects covering a broad range of healthcare issues from biologics therapies to reimbursement policies. Dr. Richer returned to McGill in 2011 as the Associate Director for the CIHR Institute of Genetics. Dr. Richer has been involved in the strategic orientation of the institute, the development of its team and the delivery of its activities. While establishing tight links with the national research community, an international focus is also infused in these three sectors. Personalized medicine and rare diseases constitute two area of focus of his work as he has been involved since 2012 in CIHR's Personalized Medicine Initiative and Personalized Health Initiative, was a member of the CSA PerMed, is member of IC PerMed and E-Rare and is involved in the development of ERA PerMed.
Maria Jose Ruiz Alvarez	She is medical doctor as degree and specialist in Clinical Microbiology. She works as researcher at the Research Coordination and Support Service of "Istituto Superiore di Sanità" in Rome. Currently, She is also supporting, as a scientific

Italian Ministry of Health, Italy	officer, the Directorate of Research and Innovation at the Italian Ministry of Health. She is the representative of the Ministry in several International projects and initiatives in the field of One Health, Antimicrobial Resistance, and Personalized Medicine. Such as the ERA PerMed , EULAC PerMed and the EU_Africa PerMed CSA where She is involved mainly in work packages on PM capacity-building. As European correspondent of ECRIN, She assists the Italy-specific Training and Education Team in identifying training needs and requirements of local network staff and clinical trials unit staff in relation to multinational clinical trials and regulatory and governance issues.
Ángel Alonso Sánchez Navarrabiomed, Spain	Degree in Medicine and Surgery, specialized in Clinical Genetics at At the health care level, Specialist Medical Genetics Area Practitioner of the Navarra Health Service (SNS-O) and Consultant Clinical Geneticist of the North East and North Cumbria NHS Genomic Medicine Centre in the United Kingdom. Leader of the "Oncogenetics and Hereditary Cancer" groups of the Biomedical Research Center of the Government of Navarra, Navarrabiomed, and of the Institute of Health Research of Navarra, IdISNA, and director of the Genomic Medicine Unit of Navarrabiomed. In the field of Personalized Medicine, he has been Scientific-Technical Coordinator of the Personalized Medicine Strategy of Navarra; secretary of the Personalized Medicine section of the Spanish Association of Human Genetics (AEGH); advisor to the Senate's Conference on the National Strategy on Genomic Medicine; member of the Executive Committee of the International Consortium of Personalized Medicine ICPerMed; and participant in the mirror groups for the 1 Million Genomes (+1MG) initiative of the European Commission.

Panel 3: Innovation System and Industrial Collaboration	
Jean-Marc Bourez EIT Health, France	Jean Marc Bourez has been appointed CEO of EIT Health on an interim basis until the end of December 2022, when a permanent CEO will be selected. Jean Marc replaces Jan Philipp Beck, who served as EIT Health's CEO since February 2018, and previously COO since 2016. Jean Marc will also continue in his duties as Managing Director of the EIT Health France Regional Innovation Hub, a position he has held since 2016. During his time at EIT Health, Jean Marc has led the strategy for EIT Health in France, as well as coordinated a number of strategic initiatives to strengthen the organisation's 'access to finance' offering including the development of the Venture Centre of Excellence and Invest Health (a newly formed commercial entity of EIT Health).
Nathalie Seigneuret IHI Europe, Belgium	Nathalie is a senior scientific project manager at IHI within the Scientific Operations team, with responsibilities ranging from the coordination of activities within the team, the management of large and complex research projects and support to public-private consortia, to engagement with stakeholders with particular focus on regulatory bodies. Nathalie joined in 2012 the Innovative Medicines Initiative (now IHI), after having worked at the European Medicines Agency (EMA) over 15 years as a scientific administrator with various responsibilities related to the development and approval of

	medicinal products. She is a state-certified Doctor in Pharmacy with degree in international drug development and Registration.
Niklas Blomberg ELIXIR, UK	Niklas is responsible for implementing the decisions of the ELIXIR Board. Niklas, a Swedish national, holds a BSc in Chemistry from Göteborg University and a PhD from the European Molecular Biology Laboratory (EMBL), Heidelberg. He is currently Principal Scientist and Team Leader in Computational Chemistry and Computational Biology at AstraZeneca R&D Mölndal, Sweden, where he has enjoyed a successful career since 1999. In addition, he has been an industry advisor in national eScience initiatives and an active participant in cross-industry research programmes.
Nataša Debeljak University of Ljubljana, Slovenia	Nataša Debeljak is an Associate Professor at the Faculty of Medicine University of Ljubljana and teaches in the field of Biochemistry & Molecular biology & Functional Genomics. As Senior Research Fellow at Medical Centre for Molecular Biology, she focuses on the molecular mechanism of erythropoietin and related pathologies, including rare blood disorder Familial Erythrocytosis. She is PI of the national research project Genetic basis of erythrocytosis in Slovenia with the aim to establish a NGS-based diagnosis of Familial Erythrocytosis. She is a mentor for PhD and MSc students. As a member of the MPN&MPNr (http://mpneuronet.com), she is cooperating with experts working in the field of myeloproliferative neoplasms and hereditary erythrocytosis.
Jens Habermann BBMRI-ERIC, France	Professor Jens K. Habermann, MD PhD, is Director General of BBMRI-ERIC (Biobanking and Biomolecular Resources Research Infrastructure – European Research Infrastructure Consortium) since September 2020. He is on leave of absence from the University of Lübeck as Head of the Section of Translational Surgical Oncology and Biobanking and as Head of ICB-L (Interdisciplinary Center for Biobanking-Lübeck). He obtained his MD training at the Medical University of Lübeck (Lübeck, Germany), received his PhD at the Cancer Center Karolinska, Karolinska Institute (Stockholm, Sweden) and a Postdoctoral Fellowship at the National Cancer Institute, NIH (Bethesda, USA). As board certified specialist in human genetics, Jens combines clinics, biobanking, and translational (cancer) research to optimise precision medicine.
Andrew Thomson EMA, The Netherlands	Andrew Thomson is a statistician at the EMA Taskforce dedicated to Data, Analytics and Methods, joining in 2014. He supports the methodological aspects of the assessments of Marketing Authorisation Applications, as well as Scientific Advice, and methodological aspects of Paediatric Investigational Plans.He has worked extensively on the methodological aspects of the EMA Reflection Paper on the use of extrapolation of efficacy in paediatric studies, and is the statistical lead on ICHE11A - Pediatric Extrapolation. Prior to the EMA, he worked at the UK regulator, the Medicines and Healthcare product Regulatory Agency. Here he worked initially as a statistical assessor in the Licensing Division, assessing Marketing Application Authorisations and providing Scientific Advice to companies. After rising to Senior Statistical Assessor, he moved to the Vigilance and Risk Management of Medicines Division, to be Head of Epidemiology. Here he managed a team of statisticians, epidemiologists and data analysts providing support to the assessment of post-licensing observational studies and meta-analyses. He also managed the team's design, conduct and analysis of epidemiology studies, using the UK Clinical Practice Research Datalink and UK

	Spontaneous Report Data (Yellow Card data). He has a PhD from the London School of Hygiene & Tropical Medicine, an MSc from University of Southampton and an MA in Mathematics from Cambridge University. He also spent 2 years working at Imperial College London, in the Small Area Health Statistics Unit specialising in the use of Bayesian Spatial Smoothers.
Maria Orr AstraZeneca, UK	Maria is currently Head of Precision Medicine for Early Oncology in AstraZeneca. In this role she is accountable for the delivery of companion diagnostics for drugs across the early oncology portfolio. In her previous role as Head of Precision Medicine for Biopharmaceuticals in AstraZeneca Maria led precision medicine activities for a diverse range of therapeutic areas including cardiovascular, renal, metabolism (CVRM), respiratory, inflammation, autoimmunity (RIA), microbial science and neuroscience. Maria is a senior leader in the field of personalised healthcare and companion diagnostic development. She has contributed to the successful launch of three personalised treatments for oncology and the delivery of over thirty companion diagnostic assays to the market to date.With over 20 years' experience in the pharmaceutical industry Maria has considerable knowledge of all phases of life science product discovery and development. Maria's extensive capabilities in the field of personalised healthcare and pharmaceutical development are complemented by expertise in project leadership, line management, high value budget administration, business development, launch readiness and commercialization. Furthermore, Maria has specialist knowledge in the areas of genetics, genomics, translational science, regulatory science, clinical development and project management as well as expertise in the fields of oncology, neuroscience, microbiology, cardiovascular disease, respiratory disease, autoimmunity and inflammation.

Panel 4: Personalised Medicine Implementation into Health Systems	
Astrid Vicente National Health Institute Doutor Ricardo Jorge, Portugal	Astrid Vicente is senior researcher, head of the Department of Health Promotion and Noncommunicable Disease Prevention at the Instituto Nacional de Saúde Doutor Ricardo Jorge, in Lisbon Portugal, and invited Associate Professor at Faculdade de Ciências da Universidade de Lisboa. She is an elected vice-chair for ICPerMed (International Consortium for Personalised Medicine) since 2017, and is the representative from the Portuguese Ministry of Health at the European 1+Million Genomes Initiative, where she is part of the Coordination Team. Astrid Vicente is also coordinating the effort to develop a strategy for Genomic Medicine in Portugal. Astrid's research focuses on understanding the etiology and biological processes underlying complex noncommunicable diseases, with a particular interest in pathologies of the nervous system, like Autism Spectrum Disorder and other neuropsychiatric disorders. Astrid is interested in a broad perspective of interactions between genetic and environmental factors to clarify the origins of multifactorial disorders, and promote progress towards translation of knowledge into personalised strategies for medical care and prevention. She holds a degree in biochemistry, and a PhD in molecular biology from Universidade de Coimbra (1996).

Toni Andreu EATRIS, The Netherlands	Toni Andreu is an M.D., Ph.D. specialised in genetics and genomics of rare diseases. He has been working in the field of neuromuscular disorders from a clear translational perspective, from basic science to the development of cell and animal models and clinical research. During his career, he has published over 180 scientific papers, numerous book chapters and supervised several PhD programs. After working at Columbia University in New York on mitochondrial disorders from 1998 to 2001, he moved to Barcelona to create the Neuromuscular Lab at the Vall d'Hebron Research Institute where he became Director of the Neurosciences Research Program and later CEO of the University Hospital of Bellvitge, one of the largest health care institutions in Spain. He has also been extremely active in the field of policy-making, and has held positions as the Director of the Spanish National Institute of Health Carlos III as well as the Director General for Research and Innovation at the Catalan Ministry of Health. Toni is the current Scientific Director of EATRIS (The European Infrastructure for Translational Medicine), a position he has held since 2018.
Ramiro Gilardino Global Health Technology Assessment Policy Lead. Pharmaceutical Executive, Switzerland	Dr Ramiro Gilardino is a physician and executive holding positions in consulting, pharmaceutical and global organizations leading Global Value, Access, Pricing and HTA Policy teams. Throughout his more than 13 years of global experience, he developed multiple tactics focused on patient-centered value analysis, outcomes research, clinical data record analysis, and economic modeling of medical technologies. He also led health policy strategies such as strengthening health technology assessment in emerging countries, patient participation in decision-making, and public-private integration to improve universal health coverage. He is an independent advisor to Decide Health®, HTA Decision Hub part of the World Health Organization, a regional advisor to the Americas Health Foundation, and has Advised on COVID health financing for the World Bank, and the Partnerships for Health initiatives. He contributed to the last 3 ICPermed Transnational Calls and to the 2019, 2020, and 2022 EULAC Permed summer school. Editorial roles include associate editor of IJTAHC, the official Journal of HTA, and part of the editorial board of ISPOR's Value & Outcomes Spotlight. Academic roles include Affiliate Professor at the Universidad de Buenos Aires, School of Public Health. Dr Gilardino obtained his MD from the Universidad Abierta Interamericana, holds a board certification in Pulmonary and Critical Medicine from Universidad of Buenos Aires, a Master's degree in Economics and Health Management at Universidad ISALUD, and a Master's of Science in Global Public Health from The London School of Hygiene and Tropical Medicine.
Terje Peetso North Estonia Medical Centre, Estonia	Dr Terje Peetso is a member of the management board of the North Estonia Medical Centre in Tallinn. Among other tasks, she is responsible for the coordination of research and innovation activities in the hospital and for cooperation with other hospitals and clinical partners in Estonia and abroad. Dr Peetso worked at the European Commission from 2003 to 2018 on digital health, tobacco control and risk assessment. Before joining the North Estonia Medical Centre, she was Head of Sector on eHealth and Ageing Policy in the European Commission's Directorate-General for Communications Networks, Content and Technology (DG CONNECT). In 2014 she was a European Union Fellow at the University of Southern California, United States of America, where her research

	focus was on the obstacles that hinder the introduction eHealth in health care systems.
Paula García ECRIN, France	Paula Garcia holds a Bachelor's degree in Chemistry Pharmacy and Biology from the Universidad Autonoma de Guadalajara (Mexico) and an International Masters of Public Health from the Ecole des Hautes Etudes en Santé Publique (France). Paula has extensive experience in international scientific cooperation and in the field of development aid. She previously worked as a project manager for the pharmaceutical industry, for the French research agency on HIV/AIDS and viral hepatitis (ANRS) and for a public health consulting firm, managing projects in developing countries. Paula joined ECRIN as a project manager in 2019. She is focused on infrastructure development projects in Europe and beyond, helping ECRIN to liaise and cooperate with other existing and upcoming research infrastructures and networks. She managed the Horizon2020 PERMIT project, and collaborates in the EULAC PerMed and EU Africa PerMed projects. She is also involved in a series of EU-funded projects focusing on infectious diseases.
Juan José Beloqui Navarrabiomed, Spain	Dr Juan José Beloqui graduated from pharmacy school and is educated as a Hospital Pharmacist, serving in several senior positions in public hospitals in Spain. He became a Board Certified Pharmacist in Pharmacology in 2016. He had the opportunity to get involved in the development of the NAGEN-1000 project, designing the first pharmacogenetic panel and learning the great possibilities of the NGS techniques. He coordinated the development of the Pharmanagen project, awarded as ICPerMed 'Best Practice in Personalised Medicine' Recognition 2021". He combines his work as a clinical pharmacist at the Mental Health Network of the Navarra Health Service with the work as head of pharmacogenetics at the Genomic Medicine Unit at Navarrabiomed.
Stefania Boccia Cattolica Del Sacro Cuore University, Italy	She is full professor of Hygiene and Public Health at the Università Cattolica del Sacro Cuore (UCSC) in Rome. She is the Director of the Section of Hygiene of the Department of Health Science and Public Health of UCSC and President of the Public Health Epidemiology Section of European Public Health Associations (EUPHA). From 2016 -to 2018 she was Adjunct Professor at the Mount Sinai Medical School, New York. In 2018 she founded the spin-off "Vihtali srl" (Value In Health Technology and Academy for Leadership & Innovation) at UCSC. In 2021, she has been nominated European Climate Pact Ambassador from the European Commission, and nominated as Italian Expert Representative for the Italian Ministry of Health for the EU project. Her main research interests concern personalized medicine policies in Italy and internationally, and the interaction between genetic and environmental factors in the risk and prognosis of gastric and head and neck cancer. In the past years she coordinated the "Personalized PREvention of Chronic Diseases" project (PRECeDI, EU-H2020 MSCA- RISE), for which she obtained a recognition from the ICPerMed Consortium.

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