2ND ICPERMED FAMILY MEETING

"STAYING CONNECTED TO SHAPE THE FUTURE FOR PERSONALISED MEDICINE"

October 19, 2023

Meeting Summary

1. Summary

The ICPerMed Family Meeting "Staying connected to shape the future for personalised medicine" took place virtually on 19 October 2023. It was hosted by ICPerMed and organised by the ICPerMed Secretariat. The meeting was moderated by the ICPerMed Chairs Ejner Moltzen, Hemma Bauer and Gianni D'Errico. Up to 60 invited participants from ICPerMed, the ICPerMed Advisory Board, the ICPerMed supporting Coordinating and Support Actions (CSAs) and invited international speakers joint the meeting and discussed the outcomes of the ICPerMed Working Groups and the ending ERA-Net ERA PerMed as well as internationalisation of ICPerMed and aspects of prevention in personalised medicine (PM). At the end of the meeting an outlook about the new European Partnership for Personalised Medicine (EP PerMed) was given and the importance of the sustainability of ICPerMed achievements was emphasized.

2. Session: ICPerMed Working Group output in support of personalised medicine implementation

Output Working Group "Clinical Studies in PM":

The session presenting the results of the ICPerMed Working Groups was opened by the presentation from Wolfgang Ballensiefen (co-lead of the ICPerMed Working Group "Clinical Studies in PM") of the ICPerMed brief "Optimizing Clinical Research for PM". This document provides a set of recommendations for policy makers, funding bodies and regulatory agencies based on the conclusions of the ICPerMed family CSA Permit and elaborated by the ICPerMed Working Group "Clinical Studies in PM". These recommendations will improve quality of PM research translating into better tailored approaches for patients and citizens, more efficient and sustainable health care services, through the optimized use of the existing resources, and improving the return of public investment in research. The importance of the right framework was discussed, as it needs high quality projects to convince regulatory authorities of PM. This document further provides helpful

recommendations for planning of transnational clinical trials. These will be needed to validate specific personalised approaches due to the low number of patients.

Output Working Group "PM in healthcare":

Monika Frenzel (lead of the ICPerMed Working Group "PM in healthcare") presented the results of the upcoming ICPerMed report "Challenges, Opportunities and Facilitators in Implementing Personalised Medicine". This report is based on the collection of PM applications and interview results. It addresses the following aspects:

- Engaging relevant stakeholders in the implementation process of PM
- Collaboration between relevant stakeholders during the implementation process of PM
- Infrastructure needed during the implementation process of PM
- Education and training in PM
- Resource allocation during the implementation process of PM
- Regulations and legislations for PM approach

The report will soon be published on the ICPerMed website.

Output Working Group "Health Economic Value of PM":

Etienne Richer (lead of ICPerMed Working Group "Health Economic Value of PM") presented the reflections of this Working Group on the value of PM from a health economics perspective. The reflections were based on the HEcoPerMed position paper "Personalised Medicine from a Health Economic Perspective - Lessons Learned and Potential Opportunities Ahead", the 3rd ICPerMed Workshop "Personalised Medicine: How to Ensure Value-based Implementation" and the ICPerMed Conference "Prelude to the Future of Medicine". The following main recommendations were elaborated:

- Integrating the societal perspective as an essential component of health economic considerations
- Considering the benefits and preferences for patients, citizens and caregivers in addition of the health gain from a healthcare perspective
- Looking at the lifecycle of the condition and long-term impact for the patient (turning the patient into a healthy citizens) – what if we act earlier in the care pathway (better frame for assessing the value of early intervention)
- Establishing new financial and reimbursement models to build a sustainable system for both the payers and for industry
- Including molecular diagnosis and biomarkers in the evaluation and value assessment to optimise the use of existing and new treatments

A publication of this document is planned for the beginning of 2024.

It was discussed how a realistic balance between benefit and costs can be better explored and outlined. Re-use of data will be an important factor influencing the health economic evaluation of PM approaches, as money will be saved and a long-term sustainability of PM approaches will become

evident. Similarly, the costs of not implementing PM should also be considered, as PM can contribute to more efficient use of resources (e.g. prescribing drugs that have no or even a negative effect). ICPerMed is not aware of any study, but these aspects might be worthwhile to be followed up from the funders' perspective. However, uptake of any new innovation needs to be justified by appropriate clinical studies. Patients and citizens should be involved to change the mind set regarding the evaluation of the cost/benefit balance. In order to make PM or even personalised prevention mainstream, citizens' needs and wishes play an important role when requesting to know their personal genetic features. This might push a broad adoption of PM approaches (e.g. pharmacogenomic approaches) in health systems.

3. ERA PerMed Results and Outlook

In this session, Cristina Maria Nieto Garcia (ERA PerMed coordinator) summarised the results and achievements of the ERA-Net on Personalised Medicine (ERA PerMed).

ERA PerMed started in December 2017 and will end in November 2023. The ERA PerMed consortium with 32 funding organisations from 23 countries organised five Joint Transnational Calls. The following main outcomes and impacts have been identified:

- A successful alignment of PM research funding
- Improved scientific knowledge and better evidence for PM for different diseases
- Implementation of a flexible call design to allow the integration of different PM key stakeholders covering the entire value chain and facilitating knowledge transfer
- Promotion of clinical research with a personalised approach considering the stratification of patients
- Awareness creation for the importance of patient involvement in research projects
- Support of the development of big data analytics tools, standards and sharing of data and outcoming results, following FAIR data principles

Due to the high interest of the research community in ERA PerMed, it is highly appreciated that the activities of PM research funding will be continued in EP PerMed.

4. Session International Perspectives on Personalised Medicine

The session "International Perspectives on Personalised Medicine" had two geographical foci, namely East Africa and Latin-American and Caribbean countries.

Focus East Africa:

In the first talk, Teresia Nyawira from NACOSTI reported on the status of PM in Kenya and the impact of the collaboration between Africa and Europe on the national strategy. In conclusion Kenya has the capacity to implement PM, but needs to take some action including the following:

- Accompanying support with resources and a think tank to steer PM programme
- Promote awareness and citizen education; strengthen ethics and regulatory framework

- Promote Omics' science – large cohort studies and harness big data sets, set up national biobanking facility/strengthen institutional sample repositories and export control, identify diseases of focus, utilize e-health/records/platforms
- Human capacity development with relevant skills – molecular/clinical geneticists, genetic counsellors, bioinformaticians, data scientists
- Strengthen data Infrastructure - data generation, mining and translation into clinical care, storage, sharing frameworks

In the discussion it was outlined, that the project EU-Africa PerMed has supported the progress of the PM agenda in Africa, because it brought stakeholders together and created awareness.

In the second talk, Evelyn Gitau from APHRC, gave an overview about challenges and opportunities in cross-border collaborations in the field of PM in the Eastern African region.

The following four challenges have been identified as relevant hurdles:

- Differences in data privacy laws and concerns about data security can hinder the sharing of patient information and genomic data across borders
- Variability in healthcare infrastructure and funding across countries can limit the adoption of PM technologies and research initiatives
- Differing regulatory frameworks for healthcare and research can impede the development and implementation of PM solutions
- Building a skilled workforce and increasing the number of healthcare professionals familiar with PM is a significant challenge

As opportunities of cross-border collaboration the following aspects have been discussed:

- Collaborative efforts can enhance disease surveillance, enabling early detection and response to regional health challenges
- Joint research projects can leverage the diverse genetic profiles and health issues in the region to develop more effective PM strategies
- Advocating for harmonised regulations and policies can facilitate the free flow of information, samples and technologies
- Sharing resources, such as genomic databases, can lead to more comprehensive and accurate diagnostics and treatment options
- Engaging with international organisations and donors can help secure funding and technical assistance for PM initiatives

Focus Latin American and Caribbean countries:

In the first talk, Joaquin Guinea (EULAC PerMed WP2 Lead) provided insights into the mapping results of PM approaches in the CELAC region. Currently, as such PM is not a priority area in health research and innovation policy in Latin America and the Caribbean. Specific PM funding programmes are only abundant in Brazil, Mexico, Argentina and Cuba. For the region the following future perspectives were summarized:

- Increase the participation of LAC institutions in PM-related clinical trials
- Strengthen the research capacity from a technical perspective
- Make costly innovative treatments accessible to the population without risking the sustainability of the health system
- Develop coherent and comprehensive national PM strategies

Reason for the lack of funding of PM does not seem to be a general lack of interest in PM. Because the EULAC PerMed project ran at the peak of the COVID crisis, the focus of the countries was not on PM at this time. However, some success stories could be identified, on which to further build on.

In the second talk, Diadelis Remirez Figueredo, Vice President of the Cuban Society of Pharmacology, gave an overview about activities of the National Group of Personalised Medicine in Cuba and the RELIVAF initiative. In Cuba, medical services are totally free of charge for the citizens. Therefore prevention, early diagnosis and a rational use of medications is critical. The RELIVAF initiative aims to address pharmacogenetic and/or pharmacogenomic research to understand mechanism of actions of different drugs. Examples are studies of genotype polymorphisms for lung cancer in the Cuban population. The publication record of the initiative was summarised. A creation of a Latin American pharmacogenomics platform and database is foreseen in 2024.

In the overall discussion, the importance of training and the generation of real-world data and real-world evidence was highlighted for the advancement of PM in these countries.

5. Personalised Medicine prevention: Presentation of the concept paper/SRIA of the CSA PROPHET look

In this session, Roberta Pastorino, UNICATT, presented the first results of the CSA PROPHET, which is the youngest member of the ICPerMed Family.

The overall objective of the CSA PROPHET is to co-create with stakeholders a Personalised Prevention Roadmap for the future healthcare, in order to support the definition and implementation of innovative, sustainable and high-quality personalised strategies that are effective in preventing chronic diseases. The PROPHET CSA agreed on the following definition of personalised prevention:

"Personalised prevention aims to prevent onset, progression and recurrence of diseases through the adoption of targeted interventions that consider the biological information (e.g. genetic and other biomarkers, demographics, health conditions), 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"

PROPHET is revolving around the Stakeholder engagement and the Strategic Research and Innovation Agenda (SRIA) development. There are three main strands of activities: Mapping, Assessment, and Building. First mapping results have been presented. The publication of the SRIA is planned for September 2025. In the meeting, it was discussed how the reimbursement of preventive approaches can be considered and an exchange with the European Partnership Transforming Health Care Systems was proposed. The importance of dissemination of the CSA results was outlined, as citizen and patient engagement is crucial for successful instalment of preventive measures.

6. Input of the ICPerMed Family to EP PerMed

After a short review about the last ten years of supporting PM research in Europe by the ICPerMed Family, the Strategic Research and Innovation Agenda for PM was introduced by Monika Frenzel. Then, Wolfgang Ballensiefen provided insights in the planned activities of the European Partnership on Personalised Medicine, EP PerMed, giving an impression about the PM roadmap for the next ten years. 51 partners joined EP PerMed investing ~370 Mio. Euro in the next ten years in seven Joint Transnational Calls and additional measures such as WP-related calls, events and activities to support PM research, innovation and implementation. EP PerMed is structured in five work packages:

- WP1: Coordination and Management
- WP2: PM Research-related Funding and Support
- WP3: Accelerating PM Development, Innovation and Absorption – Maximising Impact
- WP4: Implementation of PM in Public Healthcare
- WP5: International, Transnational, Interregional and Overarching Cooperation

It was outlined, that ICPerMed will continue as international communication and exchange platform on PM research, funding and implementation and will be supported by WP5 of the Partnership. In future, ICPerMed will leverage the work of the CSAs and explore more interactions to increase the outreach and integration of further international partners. New international ministries and funding organisations need to be actively included into ICPerMed to increase the knowledgebase and exchange experiences worldwide. The aspect of internationalisation should be integrated in the next EP PerMed SRIA version.

7. Outlook

Especially in times of accumulating international political crises, international research collaborations are important initiatives to be examples how can constructively worked together for the benefit of citizens and society. In this line, ICPerMed will continue to reach out to new international partners, to strengthen the communication and to foster collaboration in the field of PM. Even if most of the CSAs of the ICPerMed Family have ended or will be ending soon, ICPerMed will offer a platform to collect the CSAs' results and make them available. In collaboration with EP PerMed, ICPerMed will continue to form a suitable framework in terms of infrastructures, resources and regulatory procedures to foster the development and implementation of PM.

ANNEX

AGENDA

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19 October 2023

12:30 **Introduction and welcome by ICPerMed Chair**
Ejner Moltzen

12:35 **Welcome by the European Commission**
Carmen LaPlaza Santos

12:40 **ICPerMed Working Group output in support of personalised medicine implementation**
Session Chair: Hemma Bauer (ICPerMed Vice-Chair)

Presentation of the ICPerMed brief "Optimizing Clinical Research for PM" (15')
Wolfgang Ballensiefen (ICPerMed WG1 co-lead)
Presentation of the ICPerMed report "Challenges, Opportunities and Facilitators in Implementing Personalised Medicine" (15')
Monika Frenzel (ICPerMed WG2 lead)
Presentation of the ICPerMed strategic document considering HECoPerMed achievements (15')
Etienne Richer (ICPerMed WG5 lead)

Open discussion on further CSAs' results and possibilities to make those available/usable in the future (35')
Moderation: Hemma Bauer (ICPerMed Vice-Chair)

14:00 **Presentation of ERA PerMed results and outlook**
Maria Cristina Nieto Garcia, ISCIII, ERA PerMed coordinator (30')

14:30 – 14:45 Break

14:45 **International perspectives on Personalised Medicine**
Session Chair: Gianni D'Errico (ICPerMed Vice Chair)

African-Europe collaboration in PM – focus on East Africa – country and regional perspective (20')

1. Policy perspective: Status of PM in Kenya and impact of African/European collaboration/exchanges in the field of PM on the national strategy (10')
   Teresia Nyawira, NACOSTI, Kenya
2. Cross-border collaboration: Strengthening the collaboration in the field of PM in the Eastern African Region - challenges and opportunities (10')
   Evelyn Gitau, APHRC, headquarter located in Kenya

Perspective of Latin American and Carribean countries on personalised medicine (20')

1. Results of EULAC PerMed Work Package 2 (10')
   Joaquin Guinea, EULAC PerMed WP2 Lead

2. Activities of the National Group of Personalised Medicine in Cuba and the RELIVAF initiative (10')
   Diadelis Remirez Figueredo, Vice President of the Cuban Society of Pharmacology

Panel discussion - International perspectives on PM – Lessons learned and future perspectives (35')

Moderation: Gianni D'Errico (ICPerMed Vice Chair)

Panellists: Teresa Nyawira, Evelyn Gitau, Joaquin Guinea, Diadelis Remirez Figueredo, Ejner Moltzen (as ICPerMed Chair and Sino-EU PerMed ambassador)

Wrap up of discussion

Gianni D'Errico (ICPerMed Vice Chair)

16:00 – 16:15 Break

16:15 **Personalised Medicine prevention: Presentation of the concept paper/SRIA of the CSA PROPHET (30')**

Roberta Pastorino, UNICATT (on behalf of the PROPHET coordination)

16:45 **Input of the ICPerMed Family to EP PerMed**

Welcome: Ejner Moltzen (ICPerMed Chair)

Information about the EP PerMed preparations (e.g. SRIA) and activities (40')

Wolfgang Ballensiefen, DLR (EP PerMed coordinator) & Monika Frenzel, ANR (EP PerMed co-coordinator)

Q&A of CSAs to EP PerMed (20')

Wolfgang Ballensiefen & Monika Frenzel, all participants

Moderation: (Ejner Moltzen, ICPerMed Chair)

17:45 h **Final discussion and summary of the meeting**

Ejner Moltzen, ICPerMed Chair

18:00 h **End of ICPerMed Family Meeting**