EP PerMed
European Partnership
for **Personalised Medicine**

1st EP PerMed Round Table

De-risking Innovation in Personalised Medicine

27.05.2024

Venue: EIT Health House, Guimardstraat 7, 1040 Brussel, Belgium

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EP PerMed
European Partnership
for Personalised Medicine

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This report represents the views and opinions of the experts and stakeholders involved in the consultation processes organised by EIT Health on behalf of the European Partnership of Personalised Medicine (EP PerMed). The insights and recommendations presented are based on the discussions and interviews conducted with experts and stakeholders before, during, and after the round-table event. The findings in this report were further informed by EP PerMed partners and other data collected up until October 2024. Therefore, some content may reflect the state of knowledge and context as of that time and might not align with subsequent updates or developments.

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1 European Partnership for Personalised Medicine (EP PerMed) Round Table Events

Over the next decade, Personalised Medicine (PM) is expected to make a significant contribution to health care by tailoring the right preventive or therapeutic strategy to the right person at the right time. It will also improve health outcomes considering sustainable healthcare systems through better integration of research, development, innovation and implementation of PM for the benefit of patients, citizens, and society.

This involves not just research achievements and only advancement of novel technologies, it also involves new ways of working, adaption of frameworks and decisions for individuals, organisations, networks, policy and communities such as new ways of managing PM related and utilising data, and creates new opportunities for PM innovation as well as health and care and economic growth.

Therefore, EP PerMed is organising a series of annual round table events over the next few years which will take place in seven different geographical locations with PM dedicated topics.

The aim of the round table series is to:

- Increase visibility and awareness of Personalised Medicine (PM), especially in a regional context
- Assess readiness of research, innovation, and healthcare systems to implement PM, by identifying and validating (potential) general developments as well as drivers and hurdles
- Suggest concrete objectives and actions, to accelerate PM development, innovation, and implementation

The first discussion took place in Brussels, on Monday 27 May 2024 before the Belgian Presidency event “Converging Technologies towards Healthcare of the Future” and centred on "De-risking Innovation in Personalised Medicine", with a focus on the Netherlands and Belgium.

Organised by EP PerMed Partner, the European Institute of Innovation and Technology in Health (EIT Health), the event was attended by industry leaders, clinicians, healthcare providers, patient advocates, policy makers, government agencies, data specialists, researchers and academics.

2 Participating Stakeholders

- **Jerome van Biervliet**, Managing Director, VIB and Keynote Speaker
- **Einer Moltzen**, International Consortium for Personalised Medicine (ICPerMed) Chair and Keynote Speaker
- **Sofie Bekaert**, Head of Program Health, King Baudouin Foundation
- **Montserrat Daban Marín**, Director of Strategic Foresight and International Relations, Biocat
- **Elfride De Baere**, Senior Full Professor, Ghent University Hospital
- **Marc Dechamps**, Chief Executive, Bioxodes SA
- **Isabelle Francois**, Director Innovation & Strategy, Medvia
- **Jens Habermann**, Director General, Biobanking and Biomolecular Resources Research Infrastructure – European Research Infrastructure Consortium (BBMRI-ERIC)
- **Pieter Jacobs**, Head of Manufacturing Sciences & Technology (MS&T) Europe, Legend Biotech
- **Marc Van Den Bulcke**, Head of Service, Belgian Cancer Centre, Sciensano
- **Bart Haex**, Director Business Development and External Relations, EIT Health BeNe (Speaker and moderator)

3 Executive Summary

During the first section of EP PerMed roundtable, participants discussed the need for **future talent** across all aspects of PM, from research and technology to implementation. They agreed there is a critical need for a long-term vision and investment in training and education, both in relation to PM research, technology and implementation (e.g., data managers), in relation to operations (e.g., biomanufacturing), as well as management (e.g., entrepreneurs). In the dynamic field of PM, where new discoveries and technologies are constantly emerging, we have to embrace lifelong learning (e.g., reskilling or upskilling) as a cornerstone of professional development and patient care. Furthermore, digital health literacy is needed to ensure effective communication among all stakeholders, which will help make a real impact.

During the second section of EP PerMed roundtable, participants discussed the role of supporting **infrastructures**. Efficient generation and utilisation of data plays an important role in realising the benefits of PM, and therefore data harmonisation, interoperability, security, avoiding duplication and facilitating access to existing resources are crucial to support PM. Solutions should fit into everyday treatment and diagnostic processes, and should be convenient and fast for healthcare providers to use. Investments and incentives are needed to foster these new ways of working.

With regards to funding, member states and the European Commission need cooperate and align as stakeholders and look at the whole picture, discover where the actual gaps are, ask what is really needed, what the priorities should be, and whether the gaps can overcome by existing or new initiatives or collaborations. It is not just about having infrastructures, but making them more visible and more agile in data sharing (e.g., through a market-place with harmonised processes and transparent pricing for data access) to help companies innovate and bring solutions into the market.

Collaboration and stakeholder engagement are vital for the successful development and implementation of PM: collaboration between education and industry; collaboration across disciplines, and across regional, national, European and international levels; collaboration that involves patients, citizens and healthcare providers. The round table highlighted the importance of a coordinated effort to address the challenges and opportunities to de-risk innovation in PM. By fostering collaboration, investing in talent and infrastructure, and adapting common regulatory frameworks, Europe can lead the way in personalised medicine innovation and implementation.

4 Talent and Skills for Personalised Medicine

During the roundtable, participants discussed the need for future talent across all aspects of personalised medicine, from research and technology to innovation and implementation. What are their needs relating to new ways of working and the widespread adoption of PM? How to overcome the boundaries between providers, researchers, industry and patients to get this done?

4.1 The need for talent

From an EU perspective, simply searching for talents is a short-term fix, what is needed, is a long-term vision and significant investment in training and education.

PM has changed the professional landscape of healthcare and brings many new opportunities for a broad spectrum of talent, complementary to physicians and healthcare professionals, to create and innovate within and beyond the sector. Increasing visibility of available roles including scientists, engineers, bio-informaticians, laboratory experts, data scientists, and people to translate the science breakthroughs to the patients and citizens, will help promote and attract these skills and professions to a wider audience.

PM approaches allow for the identification of several key skills currently needed in research, development and production. For example, industrial quality control requires a tremendous number of qualified non-PhD personnel, and although an appropriate number of PhD and doctors are trained each year, the healthcare system needs might not be fitting with their expectations and training. Ultimately therefore, the goal should be to encourage the education systems offer to match even better with healthcare

needs. This should foster better fitting jobs, essential for health workers fulfilment, which in turn is necessary to maintain their motivation to continue training in a fast-moving environment.

In the dynamic field of PM, where new discoveries and technologies are constantly emerging, healthcare professionals need to embrace lifelong learning as a cornerstone of professional development and patient care. This commitment to continually updating their knowledge and skills with ongoing involvement in research and education ensures that patients receive the most current and effective treatments tailored to their unique genetic profiles, which helps improve outcomes and revolutionises the healthcare landscape. However, despite the well-established systems for Continuous Medical Education, the lack of availability of updated and adapted trainings in for example PM approaches reduces the capacity of regularly updating the workforce with state-of-the-art developments.

The goal should be to think holistically, with the health care system viewed as a continuous chain from research to the patient, instead of simply the place where the care is delivered.

> "I lecture at Ghent University, and teach a course on innovation management which opens up the ecosystem to students as future healthcare innovators.
>
> Every year I arrange an interactive session where I ask the students to choose an app and then discuss whether it adds value. They **always** say that in general it's a nice business idea, but there is no value towards the common good.
>
> I think this kind of reflection and system-thinking is needed in training programmes. We're all trained in hard-core R&D, we know what lab, development, and valorisation is all about, but the true valorisation is putting it into perspective in the broader system, looking at utilisation, and how will it be used beyond the digital savvy or quick adopters."
>
> **Sofie Bekaert**

4.1.1 Closer collaboration between industry and universities

Many companies have specialised in-house training departments for various fields relevant for PM, but it takes time to get talent up-to-speed.

To ensure relevant skilled talent is available to support growing companies, there needs to be closer collaboration between universities and industry in general, including different types of companies, such as start-ups and more established companies. These partnerships will help establish the right curricula that universities and colleges need to implement to support building and closing the knowledge gap.

4.1.2 Training PM entrepreneurs

The innovation process, which validates and implements an idea, e.g., by moving it forward to the point of having sufficient evidence to go to clinic and develop a product, is one aspect. Another important aspect that start-up companies often struggle with is the right skills to become successful entrepreneurs, first-time CEOs, or members of management teams. A great scientist doesn't necessarily equal a successful CEO. Therefore, it's vital for PM entrepreneurs to be able to develop the knowledge and skills needed to understand all the elements and challenges of development of a company, including technical development, clinical development, fundraising, governance, and everything related to company management.

4.1.3 Breakout

VIB Grand Challenges Program

Patient feedback is highly valued by researchers. It helps shed light on various aspects that have escaped the science theory, such as impact on quality of life, non-standard symptoms, etc.

VIB is dedicated to fostering excellence in the field of life sciences and leveraging research outcomes for economic growth. Its Grand Challenges Program is a translational research program that aims to increase the societal impact of VIB's research. The programme teams up VIB researchers with experts from outside VIB who have complementary expertise, e.g., clinicians, payer organisations, patient families, etc., to help generate new, otherwise untapped avenues that can create added value for society.

These dialogues between clinicians, researchers, patients and their families discuss, in depth, the solutions being considered and how it came about in Flanders. The objective of these sessions is information sharing, and very often, questions and views are raised from the different participants that has nothing to do with the 'science part'. This is inspiring for researchers, who may not be aware, in practice, what the actual patient needs are, despite having worked on a certain disease for many years. This offers a new and valuable level of 'energy' into the whole research and development of PM to create a

concept that's fit for purpose; one that starts with the needs and is adapted to the situation.

One example focused on personalized medicine (PM) for rare immune deficiency diseases. While scientists were working on improving immune profiling and off-label treatments, the real issue identified through conversations with the affected children was a lack of understanding from their friends and teachers about how much the disease impacted their daily lives. The children struggled to explain the severity of their condition to their schools and teachers. To address this, VIB created a documentary that followed the lives of a young child and a teenager, which was shared internationally to raise awareness and foster understanding. Both the parents and the children were very pleased with the results (**https://piddocu.sites.vib.be/en**).

4.1.4 Repurposing skills

We have a shortage of medical personnel, and they can work in the health care system only if they have a recognised qualification. The complicating factor is that within Europe, there are healthcare professions that are recognised in some countries but not others. "Pact for Skills", one of the flagship actions of the European Skills Agenda initiated by the European Commission (EC), and developed by Directorate-General Employment, Social Affairs & Inclusion, aims to support public and private organisations respond to the needs and challenges faced by the health care sector and establish a shared model for skills development in Europe, pooling knowledge, experiences and resources. The "Skills Partnership for the European Health Industry", coordinated by EIT Health, aims to attract new talent and support the existing workforce through reskilling and upskilling initiatives, promoting learning opportunities and 'on-the-job' training that strengthen the sector's resilience and equip professionals with the skills they need.

With an increasing number of artificial intelligence (AI) consortia being formed, such as TEF Health (**TEFhealth.eu**), there could be opportunities to educate more data scientists. However, when it comes to operational profiles, this could be a task for regional university colleges or affiliated training centres to offer upskilling and reskilling courses so that, for example, a nurse can learn to perform new operational tasks.

Additionally, there is a need for all healthcare professionals to improve their knowledge so they are can critically look at the new PM innovations and technologies being developed. In this way they can be an active partner in the development and improvement of fit-for purpose PM approaches.

4.1.4.1 Upskilling/Reskilling

Upskilling: The process of learning new skills or enhancing existing ones to stay relevant and competitive in one's current job role. It's about personal development within the context of one's current career path.

Reskilling: The process of learning new skills for a different job role, often within the same organisation but sometimes in a different sector. This is essential to attract professionals to the healthcare sector and particularly to PM.

Both upskilling and reskilling are crucial for career growth and adaptability in the ever-evolving job landscape.

4.2 Future needs and skills related to the adoption of Personalised Medicine

Healthcare systems do not only include healthcare professionals, but also engineers, data and IT experts, rules and regulations, ethical and social experts, and patient organisations, all working together to make the systems more patient-centric. Therefore, the goal should be to think holistically, with the health care system viewed as a continuous chain from research to the patient, instead of simply the place where the care is delivered.

From a government perspective, there is a lot of investment needed, so it's important to determine which area(s) to prioritise.

From a population perspective, education and information is required to bring about societal change where the whole population embraces the concept of PM.

From a health literacy perspective, in relation to PM health literacy is lacking in patients, citizens, and also in the caregiving population, and will therefore need a systems approach to improve this, with all players and people from different backgrounds and positions all working with the same shared strategy.

4.2.1 Health Literacy Skills

Breakout Box: Health literacy is the ability to understand, access, and use information about health to make informed decisions and take actions that promote well-being.

Health literacy needs to be integrated into every aspect of personalised medicine

Patients, citizens, care givers and healthcare professionals could be better informed about the meaning and relevance of PM. While healthcare professionals don’t need expert knowledge, it’s imperative they are adequately familiar with the concept to help patients and citizens understand what PM is all about.

Specialist, e.g. oncologists, are more aware of PM concepts and how to implement them in their practices than general practitioners (GPs). However, GPs are more likely to be in a position to educate the citizen and patients about PM due to their geographical and social proximity with the population. Fortunately, with the advance of medical technology making its way in to the doctor's office, citizens and patients will gain visibility of PM concepts and implementation.

Additionally, PM requires adaptation of the healthcare reimbursement structure to incorporate the need to build evidence continuously as new PM approaches are implemented. As well as efficient heath technology assessment models that can balance between the advantages of the personalisation of care versus the cost.

4.2.2 Building trust and transparency around data

Utilising structured high quality health data is essential for realising the full potential of PM. By pooling clinical, biomarker and genomic data as well as lifestyle and environmental data researchers and innovators can uncover patterns and correlations that would be impossible to discern in smaller datasets. Efficient use of health data in a collaborative approach will accelerate research and innovation such as the discovery of biomarkers for disease susceptibility and drug response, leading to more precise diagnostics and effective treatments.

While the research community recognises the value of data sharing in advancing a comprehensive understanding of health and disease, which in turn drives innovation and enhances patient care in PM, there is still a lack of widespread awareness among the general public.

The King Baudouin Foundation ran a survey with around 2000 Belgian patients and citizens, along with an additional survey across Europe, reaching approx. 30,000 people, on their knowledge regarding data and health data sharing. The results were very clear. In terms of everything that deals with sharing of evidence and data for the 'common good', it was GPs who were the most trusted. But the question is, does their current training support personalised medicine, health, data, and digital literacy?

With the current, on-going data access challenge, there's a need to talk about fair data solidarity which would not only help improve innovation but add value to society.

In general, building trust and transparency around data sharing and access is important for all stakeholders, especially the patients, and there are two potential opportunities:

1. Introducing incentives for non-sensitive data and information sharing along the healthcare system chain, including biomedical and patient research data. Data sharing at university level brings in core funding and other benefits, but if changes aren't made at this level, scientists and clinicians might be reluctant to share further along the chain for fear of losing control over what they are doing and where they are in a good position to attract further funding, etc. And this means that all the networks and infrastructures for data sharing and accessibility might not make any difference.
2. Start a dialogue on the unmet needs and what value a solution can bring to address those unmet needs. In clinical trials there is a lot of effort made in informing patients, getting informed consent, and so on to include them in the trial, but rarely are the

results of the trial shared with them. Such a continued dialogue may help to gradually increase the need-solution fit.

4.3 Overcoming the silo issue

4.3.1 Cross Linking the Regional and International Arena in PM Innovation and Implementation

There are important EU research initiatives, such as the Innovative Health Initiative (IHI), a public-private partnership funding health research and innovation, and Horizon Europe which is the key funding programme for research and innovation.

For PM implementation, a possible option is to be small, agile and work as a beehive works with everyone having a specific task in a specific domain, along with adaptable and flexible pre-defined processes of PM that involve all stakeholders, from patients to industry.

In Belgium, for example, Vaccinopolis, UZA and University of Antwerp started an initiative where human health studies on viruses and infectious viruses, can take place in a controlled environment, in Antwerp or Brussels. For the first time, ethical communities composed of experts from different nationalities are judging all cases, rather than institute by institute. This clustering is something that could be done for many other diseases.

The keys to making this work are the acceptance of specific expertise and competencies of regional clusters and the willingness to share the knowledge and data.

4.3.2 Stronger Collaboration Across Regions

The new European Innovation Agenda and initiatives like the European Health Data Space (EHDS) are instruments that offer more opportunities for closer collaboration across organisations, regions, and borders. However, it is still a work in progress, e.g. on aligning policies to the gaps identified, e.g., in the EHDS, PM, etc., and on ensuring that Readiness Levels in all European countries to embrace these initiatives are similar. Understanding how to implement policies into practice also requires harmonised thinking within an international and European Union context, especially when it comes to setting priorities, which should consider accessibility and equity upfront

Closer collaboration is needed to bring generalised models, best practices, and to ensure that the policies implemented are relevant to reduce the gap, because most of the time the challenge is at the decision-making level.

4.3.2.1 Best Practices Examples for PM

The King Baudouin Foundation has devised a set of eight principles for caring technology *[see Appendix 1]*, that, when followed, guarantees active engagement with different stakeholders. If principles like these could be embedded in any development or funding trajectory, it would increase the probability of patients, citizens, or healthcare professionals feeling empowered and becoming engaged in the project.

4.4 Concluding Remarks

All stakeholders identified a need for a range of talents related to PM research, technology and (clinical) implementation, e.g., scientists, IT Specialists, medical laboratory technicians, data managers, experts on regulation. From an industry perspective, there is an additional need for operational and quality assurance talent, and a request for building related training into existing curricula, e.g. basic knowledge of bio manufacturing.

Collaboration within the PM value continuum is key. Making sure that the remaining silos are being dismantled, the right unmet needs are being addressed by the relevant stakeholders and more importantly, a favourable environment is created where the correct funding and regulatory approvals can be implemented quickly.

- A continued dialogue between educators and industry is mandatory to remain in sync. It is vital to understand the profiles of the talent and skilled personnel needed for PM, whatever the role or function so a real dialogue can be had with universities and education centres about new curriculums and bridge the gap between education and the reality of the field, so talent is available and ready to take on attractive job opportunities in this field.
- Collaboration across disciplines, while including patients, is crucial at regional, national, European and international levels. Digital health literacy is needed to ensure effective communication among all stakeholders, which will help make a real impact. This is essential in both education and work, considering equity and other factors. Europe is already streamlining processes across different countries and regions, which is proving to be effective. And while local implementation is necessary, there needs to be a unified effort supported by member states and regions.

In five to ten years, the generation currently being trained will face PM technologies that haven't yet been developed, and many everyday tasks will be supported by Artificial Intelligence (AI), or other technology. Therefore, to reduce the talent skill gaps it is important to create and train generations to solve complex problems, be more collaborative, and work with diversity.

5 Supporting Infrastructures

During the second roundtable session, participants moved on to discuss supporting infrastructures for PM in the BeNeLux region and across Europe, specifically around the topic of data access and sharing along with the need to ensure sustainable infrastructures and the mechanisms needed to work more collaboratively.

5.1 Data Infrastructure

For all aspects of PM, research, innovation, and implementation, it is crucial that data access needs, including biobank requirements, genomic and molecular diagnostic as well as clinical data, at local, national, regional, and European levels are addressed.

There are, for example, regional strategies in place for creating primary use data lakes, however, depending on the authority or body that is collecting the data, much of the genomic, image, and clinical data is still being gathered in silos. On a European level the European Health Data Space and the DIGITAL strategy has several ongoing initiatives including support for a legal framework as well as investments on infrastructures and pilots in a range of disease areas.

5.1.1 Challenges

Most data infrastructure challenges revolve around uncertainties in terms of the legal framework, interoperability and security including secure processing environments. There must be frameworks in place to ensure that patients can trust that their data is secure and will benefit themselves and others. With the recent approval of the EHDS regulation there is a need for a common vision and a clear roadmap on how Europe is going to realise the benefits of such a data space. In particular, pathways are necessary on how this data is going to be made accessible in a fair, transparent and compliant way. Additionally, in order to get the infrastructure in place, readiness level assessments of different member states need to inform the solutions to be implemented.

While the EHDS provides a framework containing the regulations to make it happen, it is important to build capacity locally, both in terms of healthcare and research, to be able to support equal access to PM. Most often, silos occur in the institutions actually gathering the data, so in a single region in Europe, there might be hundreds of silos from different hospitals gathering data, all using different Electronic Health Record (EHR) forms and systems.

Even if Clinical data is (increasingly) available along with regulations and suitable technical solutions to make it accessible across Europe, there is still a lot of work to be done on data handling, interoperability (e.g., semantics) and a significant divide in infrastructure readiness across Europe.

Data gathering, in particular, needs a joint ambitious plan from member states and regional stakeholders. Aspects to consider are:

- What type of information is needed from that data?
- How is data to be gathered?
- How is that data processed?
- How is that information managed?
- Which public bodies are going to take care of the information?
- What is the capacity for processing the data?

One particular initiative that has done a lot of work to solve these challenges is the 1 Million Genomes Initiative supported by the Beyond 1 Million Genomes (B1MG) and Genomic Data Infrastructure (GDI) projects that aims to make human genetic data and other relevant health data accessible in a federated way around Europe. By working on legal guidance, a common data handling framework and disseminating of best practices to enable access to sequenced genomes in the EU, it will make it possible for scientists and clinicians to study the genotypic and phenotypic data from a large number of people in secure processing environments.

5.1.2 What are (other) regions doing to support the collection and use of PM related data?

When talking about the fragmentation in Europe and the solutions needed, it is important to look to other regions across the world to see what they are doing to harmonise access to and the sharing of data.

For example, the Mayo Clinic in the US is integrating their platform data with data from around the world including Brazil and Europe, which constitutes a huge amount of shared patient data. They are giving access to and sharing their data, even with start-ups, which is something Europe should be thinking about when discussing future infrastructure plans. There are also examples of monetising data sharing, which is something that need to be analysed and discussed, as this needs to be managed in a way that it supports the individual and society as a whole and not specifically single private entities.

5.1.3 Healthcare is more than hospitals

A key aspect, is the need to rethink the roles of different professions and responsibilities in healthcare. Whether it is hospitals or primary care providers. In general, the public are trusting when it comes to sharing their data with general practitioners and hospitals for research and innovation if it improves healthcare.

In countries like Belgium, governments have invested heavily in building infrastructures for biobanking and data registries. But after the initial investment, responsibility is

passed on to hospitals who may have a lack of understanding on how to make these resources accessible to developers while keeping the infrastructure self-sustaining, as biobanks are a continually growing source that needs to be maintained.

The general public need to be part of these data discussions, to address these challenges. The public is willing to participate, and their input must be included in the conversation early on.

5.1.3.1 Example of lessons learned

A multi centric international trial, **Can.Heal** thought it would be a fantastic idea to create a common medical technology assessment (MTA). Discussions were held, and the partners created what they thought was a perfect MTA. However, when it got sent to the hospitals, complications arose. The Data Protection Officers (DPOs) became involved, and it became clear that they should have been included in the MTA creation from the beginning. They also noticed that not everyone was willing to do all the work described in the trial, preferring to be responsible for one part. In the end, this trial would have had 68 different MTAs, not a common one as they envisioned.

What needs to happen at the beginning of every project is having a discussion with a range of relevant stakeholders and attempt to reach common ground at the start.

5.2 Bio-manufacturing

Precision medicines targeting a smaller group of individuals requires new ways of manufacturing these advanced medicinal products:

- The treatments are highly personalised, i.e., one product per patient, which means there can be difficulties standardising the production process because the starting material is always different and may not react in the same way to the production process.
- The time frame required to create these treatments needs an infrastructure network, including shipments and production that is finely tuned to allow a rapid turnaround.
- Doctors are trained differently, and the machines available are not the same across all hospitals, modern hospitals tend to have the newest equipment and older ones don't.
- While blood collection, for example, is a standardised procedure, different companies may impose slightly different requirements, from drug to drug, including quality, which means there are still inherent differences that need to be dealt with to ensure the final drug product that is shipped back to the patient is of the best possible quality to be efficacious.

- Many hospitals are also developing their own solutions, and while there is some collaboration between hospitals and PM companies in terms of leveraging expertise, it's still very much a silo, and information sharing and learning from each other is limited.

There are two potential paths that bio-manufacturing can take, one is to build massive plants that can treat many patients from that single facility with hospitals, but here the time frame to patient is challenging. The alternative is for individual hospitals to have their own GMP (Good Manufacturing Process) facility where they produce their own drug products in smaller capacity, but there the regulatory needs per one therapy makes it extremely expensive.

5.3 Pan-European Infrastructures

The European Commission defines, evaluates and implements strategies and tools to provide Europe with world-class sustainable Research Infrastructures. Research Infrastructures are facilities that provide resources and services for research communities to conduct research and foster innovation. One of the most relevant for PM is the pan-European Biobanking and Biomolecular Resources Research Infrastructure (**BBMRI-ERIC**), an EP PerMed Partner, which connects more than 450 biobanks across 25 member states that are coordinated by national nodes. The list of biobanks contains either population biobanks or disease specific biobanks, but also national biobanks, research sample collections and repositories collected part of routine clinical procedures, etc. BBMRI-ERIC enables everything to be linked together for collaboration, harmonising, and standardising. The BBMRI-ERIC Portal Directory is a broad catalogue of biobanks with aggregated information on collections, where innovators can browse and filter by country, sample type, quality marks, International Statistical Classification of Diseases and Related Health Problems (ICD-10) and more. A negotiator platform supports researchers and biobankers in the complex communication necessary to achieve a consensus on conditions of samples and/or data delivery.

It is important to note that the European Research Infrastructure Consortiums (ERICs) are maintained by membership fees from the member states, meaning the core budget comes from Ministries that enable science, innovation and the implementation of personalised medicine, while the remaining budget comes from the European Commission. However, the opportunity to make use of all the infrastructures has not been fully leveraged, possibly through a lack of awareness and adequate data sharing processes. So, it is not just about having, or not having the infrastructures, what is needed are the resources to make these opportunities sustainable, more visible and more agile in data sharing, to help companies innovate and bring solutions into the market.

Another very relevant ERIC for PM is **ELIXIR**, a European life sciences infrastructure to access and analyse life science data, that brings together scientists from 21 countries and over 250 research institutes, and resources include databases, software tools, training materials, cloud storage and supercomputers.

ECRIN-ERIC facilitates multinational clinical research, through the provision of advice and services for the set-up and management of investigator or SME led clinical studies in Europe, also help to strengthen the capacity of the European Union to explore the determinants of diseases and optimise the use of diagnostics, prevention and treatment strategies.

There are also several other EU funded projects that create valuable pan-European infrastructure to support PM R&D, but in general, these infrastructures are supporting the public research European ecosystem. The integration of private industry in the asset sharing is a still challenging model, however much needed for innovation.

The current development on the European arena is the formation of so-called European Data Infrastructure Consortium (EDIC) building on the ERICs but with a broader aim of supporting research, healthcare and policy development. The above-mentioned Genomics Data Infrastructure works toward an EDIC as is the European Cancer Images Federation Infrastructure EUCAIM.

5.4 Funding and sustainability

After funding ends, many infrastructures have a requirement to be self-sustainable where, in theory, the fees charged for access to them should cover operational costs, and they should have a steady flow of users. In reality, however, infrastructure managers often lack the resources to run and market the access service, and the direct costs associated with access is usually more than R&D funding schemas can offer. As a result, the lower funding for infrastructures leads to subpar access services, making it difficult to meet user needs effectively. Additionally, the self-sustainability requirement and elevated access cost makes access to infrastructure unaffordable for start-ups.

The question is who is going to pay, if the legal requirement will be to provide data for secondary use? An accompanying challenge is the co-funding requirement in national and regional funding, which is difficult for innovators in academia and start-ups. For European Commission projects, co-funding cannot come from other regional or EC grants as this would be considered double-funding. Additionally, some funding applications have parts of a project that are ineligible for funding, making it hard to secure the necessary resources.

Many projects are co-funded by the European Commission on the one hand, and member states, cluster organisations, or stakeholders, e.g., universities, hospitals, on the other. While such projects foster alignment and harmonisation, they may also create hurdles. For example, when the Commission seeks initiatives to fund that will be self-sustainable after the Commission's investment ends, stakeholders have no interest to participate, either because the funding mechanism is not advantageous or the funding budget received versus the effort required is not sufficient. This will induce biases on the type of organisation and member states that participate.

It is essential for member states and the European Commission to engage in comprehensive discussions, to holistically assess the landscape, identify existing deficiencies, determine essential requirements, establish priorities, and evaluate if these deficiencies can be addressed through current initiatives or partnerships. This collaborative approach is crucial for effective governance and the advancement of common infrastructures within the European Union.

5.5 New ways of working

> "Co-funding typically comes from a collaboration, or a joint action, between the EC and member states both investing, and normally it works well. However, the EC often ends up investing much more than anticipated, even though it benefits the member states and is agreed by the ministry and all the players.
>
> There are seven joint actions under the EU4Health programme opening this year, worth 150M Euro, and is the first-time multiple projects are running in tandem, with all member states participating, including accession countries.
>
> This is the first time that so much funding has been allocated to health, and because everything is new, it's important to think of this as a learning phase. Although the interaction and platforms are in place, implementation takes time, sometimes even five to ten years for small changes to be fully realised. So, while work needs to be done to integrate partners, activities, and projects, it's crucial to set clear priorities for the next five years because without these priorities, there's a risk of not making any tangible impact, despite the large funding invested."
>
> **Jens Habermann, Director General, BBMRI-ERIC**

5.5.1 Pricing Models for Data Access

While most biobanks and data repositories don't charge research community to access their data, if requests for access increase substantially, charging a (small) fee might be necessary, to pay additional staff to cover the requests. Some health insurance companies, however, do currently charge a couple of thousand Euro to access their data, and this may be a challenge for research projects.

With all the testing and experimental facilities being built at European level, pricing is going to become even more important. What start-ups need is something like a market-place, a specific webpage, where they can search for an infrastructure and immediately get an idea of what assets are available and, more importantly, what it will cost.

But there is a huge difference between countries. Those who are used to sharing know what they need to do to provide access and can put a price on it, but for others it will be a difficult exercise, especially if they want to distinguish between a researcher and a private company.

ERICs can get a small percentage of revenue because they have a federated data access platform, that connects, for example, all the different biobanks, and acts as a matchmaker. Anyone can request access, and the ERIC then points whoever is requesting access, be it from academia or industry, to the individual biobank who can provide the relevant samples or data. It is then up to the local site to negotiate if there's a reimbursement fee involved to keep the local infrastructure alive, because the over-arching 'search and access' platform is maintained by member state budget.

It is important to understand that, because the data never leaves the infrastructure, the user is paying for the availability of the sharing infrastructure, and not for the data.

When it comes to PM, there is a lot of pressure on clinicians to communicate more and be involved with patient organisations, EU and national processes for expertise, as well as having to keep up-to-date with all the biomarker treatments, targets and therapies available. Not to mention asking for funding, training students, general administration, accepting reviewing positions for journals and calls, and taking care of the day-to-day service needs. To make this feasible, it is important to support health care providers with multi-disciplinary teams and with technology, where possible, which will require investment.

In the capacity building inside the hospital and the projects that are developing new technologies and solutions. The joint actions have been one attempt from the EC to actually give some seed money to build capacity while developing new technologies, but further investment is necessary.

5.5.2 A Federated Approach Quote

Montserrat Daban Marín, Director of Strategic Foresight and International Relations, Biocat: "When it comes to the region making data accessible on a large scale, the federated approach is really the only way forward. What is needed are harmonised ways of generating and sharing data, including samples, clinical phenotype data, genomics, or any kind of -omics. Structuring data, and then finding (open) technological solutions to connect different data-nodes is the way forward. But this needs resources bringing together, though by addressing the de-fragmentation, resources will be able to be utilised better.

Investment is also needed in high quality data generation, pre-processing and federated databases, which can then be shared and the data made accessible. The clinical data linked to genome is very important because there is a lot of attention right now about the population specificity of the genomes and there's still an important gap in Europe especially among some underrepresented populations, so it needs to be shared, which is essential for the study of rare diseases.

The health data in member states, that is in many cases the essential information, is stored in unstructured fields, in local language and automatically not AI readable, sometimes even in free text or pdf format."

5.6 Concluding Remarks

Data play an important role for realising the full potential of PM approaches, and therefore data harmonisation, interoperability, security, aligning investments and facilitating access to (existing) resources are crucial to support PM.

Database owners and data processors are often not willing to share their data, because providing access is an additional burden on top of their normal work. PM solutions should therefore fit into everyday treatment and diagnostic processes, consider the data available for patient but also should be convenient and fast for healthcare providers to use it. Investments and incentives are needed to foster these new ways of working.

Health data is owned by the patient/person and she/he decides what happens with it. Offering it anonymously to R&D helps bring new solutions to market which can improve the person having better health, but for this to work, trust is key. Federated systems could be the key, especially for multi-centric studies. With all the testing and experimental facilities being built at European level, pricing is going to become even more important. What data users (including, start-ups) need is something like a market-place, a specific webpage, where they can search for an infrastructure and immediately get an idea of what assets are available and, more importantly, what it will cost.

With regards to funding, member states and the European Commission need to sit together as stakeholders and look at the whole picture, discover where the actual gaps are, ask what is really needed, what the priorities should be, and whether the gaps can be filled with (existing) initiatives or collaborations.

6 Annex 1: Regulatory Challenges

Regulatory approval is one of the crucial challenges for PM and innovators in these areas. The sector is new and there are several aspects that bring extra complications to adjust clinical validation to the frame coming from the Medical Device Regulation (MDR) 2017/745 and the In Vitro Diagnostics Regulation (IVDR) 2017/746. Proper clinical validation is requirement for medical devices (including software) and in vitro diagnostics to get the "Conformité Européene" mark (CE mark) which certifies that a product has met EU health, safety, and environmental requirements, which ensure consumer safety, and is prerequisite to sell products on EU market.

For example, gene-based therapies are mainly developed per individual and the massive clinical validation is impossible for market authorisation because each solution is more or less unique. Another example is the genome information that is often used for disease risk prediction purposes, such as for national screening programs. Clinical validation cannot be done using traditional clinical trials with patients, so prediction algorithms are used for these 'not yet sick' persons and biobanks together with longitudinal health data is needed for validation. This is a totally new concept for clinical validation and, as yet, there are no best practice materials or clearance within the community to establish the requirements to get the CE mark and market authorisation.

There's a huge investment in research & innovation, but when it comes to regulation and validation of new PM solutions, there's always a trade-off between having a safe, trustworthy environment and the opportunities and speed for the innovations needed.

The current regulatory processes for PM seem over complicated and too expensive. In the context of PM, typically 'home brew solutions' for diagnostics and therapeutics are developed and validated, as opposed to CE Labelled solutions. However, this means the solution cannot be supplied to any other legally autonomous entity apart from the hospital connected to the 'home brew', making it difficult to scale it to other markets or produce it in industrial scale.

This is something that innovators are struggling with, and could potentially continue to struggle with for a few years.

Further regulatory adaptions and supporting services are needed in connection with personalised medicine which Europe should take the lead on.

7 Annex 2: Additional Findings

As part of the Round Table, interviews were performed with

- Jérôme Van Biervliet, Managing Director of VIB,
- Sofie Bekaert, Head of Program Health at King Baudouin Foundation, and
- Pieter Jacobs, Head of Manufacturing Sciences & Technology (MS&T) Europe at Legend Biotech

They highlighted some of the current drivers, and major and obstacles for personalised medicine innovation in Belgium, and Europe in general.

7.1 Current Drivers

7.1.1 Molecular Research and Science

To allow the concept of PM to have a real impact on a patient, it's vital to look first at the fundamental research questions, for example, how cancers or Alzheimer's disease develop and progress on a molecular, cellular, but also on an individual level. To be successful on this level, different types of expertise; clinicians, basic scientists, AI and bioinformatics, and heavy-ended research technologies need to be cooperated already from an early stage. This also needs to be considered in funding strategies on regional national and European level. However, to further accelerate PM innovations, academic communities and the early-stage biotech pharma industry need to join forces to succeed in the marked access and healthcare system implementation of PM approaches independent of the disease.

Jérôme explained that VIB has created an environment which has resulted in a cluster of accelerating biotechs that have been able to get financed. However, as a public non-profit party, VIB can only bring innovations so far. At some point it needs to be invested in from private sources, from the venture capital world, from the public markets, and this support is key if they want to continue to grow as a company in Europe, not only to reach their goal for personalised medicine but also the economic valorisation.

7.1.2 Support and collaboration

Another driver for PM innovation is continued support of basic research, not only for the development of technology or more evidence, but also to enable it to be at the forefront of bringing solutions to meet needs of the patients and healthcare systems.

Additionally, it's also as important to rethink how these needs will be captured and how PM can be organised in a participatory way. One driver for this is supporting activities that strengthen co-creation, and ensure clear and active communication with all

stakeholders, not just patients and citizens, but all the other players, to guarantee sustainable innovation. With all stakeholders involved the research, development, and implementation phases, they gain insights into the bigger system, and therefore dialogue will be more holistic around what a potential solution should be like, not just from the scientist's perspective.

7.1.3 Finding a treatment

For many diseases, e.g., cancers, cardiovascular or inflammatory diseases, there are drugs on the markets, but most of them treat primarily symptoms, that improve quality of life and extend the life of patients. But, we need to find treatments against the cause of the disease. With PM, especially with cell and gene therapy, there is the potential to provide a cure against the cause of the disease, especially for those with genetically caused diseases.

This innovation takes place on two fronts.

1. In many cases, academics set the stage with their crucial basic and clinical research efforts and achievements and then build spin-off companies to do translational research. While early-stage clinical trials are easier, once Phase III clinical trials are needed, they become very expensive and challenging to manage because additional expertise and budget is needed to further develop innovations. This is generally the point when industrial partners come on board.
2. For the second part of PM innovation the pharma and biotech industries need to invest in R&D, for example to identify and analyse new diagnostic and therapeutic targets, and creating production processes and, as such, creating new personalised medicine therapies aim to be commercialised.

In the past, academic institutions would complete the basic research which would then be picked up by companies. But over the last 10 years, there has been a shift to academic institutions and active investment in clinical and basic research and how to use the acquired knowledge to create new diagnostic approaches, as well as inventions and drugs. These innovation driven medical doctors and entrepreneurial scientists create spin-off companies around these new therapies and technologies which they can build and scale without the help of the big pharmaceutical players. Early funding comes from 'family, friends and fools' (FFF), then national and regional funding schemas, and then if the ideas are validated, investors consider funding.

7.2 Major Challenges

One major universal observation is the tendency to focus on the high-tech aspect without considering the various stakeholders' perspective, or more importantly, their actual needs.

The European Commission has done a great job in developing programmes within Horizon Europe that focus on stakeholder co-creation, such as the Innovative Health Initiative (IHI) whose core goals are to translate health research and innovation into tangible benefits for patients and society, and ensure that Europe remains at the cutting edge of interdisciplinary, sustainable, patient-centric health research. This makes sense at both European and regional level because a lot of stakeholder work doesn't always succeed, due to proof of value studies that map all end users' (patients, doctors, process owners, hospital admin, health funders, etc.) needs and expectations being very rarely undertaken. In many cases only citizen engagement is planned for, with the other end-user groups excluded from the study design.

To be able to take innovations to where they can really make an impact and a difference, it's important to have a bigger knowledge base, more evidence, shared best practices on how to, and what is the best way to engage stakeholders to increase trust, and the means and approaches to guarantee that the innovation might be taken up in a more sustainable way.

The communication should be done in simple language so patients, their family members and care givers understand the value of new approaches.

8 Annex 3: About EP PerMed

EP PerMed as a **coordination platform** brings together European as well as international, national and regional ministries and funding organisations, agencies and authorities, **joining forces on several levels and sectors**.

To realise the full potential of personalised medicine (PM), EP PerMed supports the **entire PM value continuum** by a wide range of definite actions, such as joint research funding and supporting activities, partnering and tailored tools, to accelerate PM innovations, which are completed by specific activities to support PM implementation. All activities are accompanied by supportive conditions regarding policy and regulatory frameworks, end-user engagement, education and knowledge exchange, aligned strategies and priority settings. The 1st **Strategic Research and Innovation Agenda for Personalised Medicine** (SRIA for PM, 2023) is guiding the tasks and actions to enable more PM and personalised prevention strategies.

The objectives of EP PerMed are:

- Putting Europe at the forefront of research and innovation through the support of multidisciplinary actions open to international cooperation.
- Translating basic research into clinical applications that make a difference for patients, their families and healthcare professionals.
- Integrating big data and digital health solutions in research and personalised healthcare.
- Strengthening the European healthcare industry and accelerating the uptake of personalised medicine solutions.
- Providing socio-economic evidence of the feasibility of personalised medicine approaches for its uptake by sustainable healthcare systems.
- Developing appropriate ecosystems for the implementation of successful personalised medicine approaches and a swift uptake of relevant innovations by healthcare systems.
- Improving health outcomes for citizen and patients and ensuring a wide access to advanced personalised medicine intervention approaches to all.
- Establishing a European national and regional network of research and innovation systems dedicated to personalised medicine.
- Filling scientific knowledge gaps, producing evidence and developing guidance and tools in priority areas for the development and the deployment of personalised medicine.

9 Annex 4: King Baudouin Foundations "Eight Guiding Principles for caring technology"

When using technology to improve the health-related quality of life of people in their daily lives, it is important to ensure that the following guiding principles are in place to guide our actions:

Promoting human technology and data management at the service of the citizen

1/ Ensure that technology and data use retain a facilitative and supportive role, serving people and society well. Maximize the opportunity for citizens to make their own decisions based on their needs for assistance and care and their health wishes.

2/ Encourage ongoing collaboration among all stakeholders by creating an integrated technology ecosystem in which interoperability, standardized protocols and core open-source technology are a given. To help patients and citizens participate optimally in the development and deployment of this ecosystem.

3/ Provide honest, reliable, transparent and understandable information on health care and health innovations. Ensure that individuals can make autonomous and informed choices (true consent) by objectively depicting the usefulness, applicability, advantages and disadvantages of innovations. People must be able to trust the products they adopt.

Support the societal anchoring

4/ Strengthen the confidence of individuals and organizations in the use of data and the design of innovations that exploit it by ensuring their ownership of their own data. Helping citizens to share their data securely and to use it as a lever for their personal well-being and for the public interest.

5/ Promote technological literacy, health skills and participation of all citizens. Commit to lifelong learning for all. Ensure that everyone is involved, including the vulnerable and disadvantaged and those requiring special attention. Innovation must focus on reducing the digital and health gaps, not contributing to their widening.

Encouraging participatory governance

6/ Develop participatory and adaptive governance of the innovation system. Encourage citizens and stakeholders to become actively involved. Adjust policies flexibly, yet vigorously, on the basis of data, experience, evidence and growing expertise.

Monitor the quality and consistency of the system

7/ Develop quality assurance systems for the innovation process, i.e. before, during and after technology development, data use and technology implementation. Control should cover content, security, transparency of information, traceability, usefulness and

efficiency. Lessons from experience must go hand in hand with scientific evidence. Introduce quality labels and disseminate the results of monitoring and evaluation.

8/ Monitor actions and check that they remain consistent with the objectives set for health and care in a broader framework of prevention, ethics and sustainability. Integrate sustainability objectives and appropriate ethical principles (e.g. human rights) into the path of innovation growth.

* As a starting point, the term "personalised medicine" itself needs to be defined as this determines future fields of actions and responsibilities. The work of ICPerMed is based on the definition of personalised medicine given in the European Council Conclusion on personalised medicine for patients (2015/C 421/03).

It states "[…] that it is widely understood that personalised medicine refers to a medical model using characterisation 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."