Personalised medicine – Region specific needs and barriers

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Jolien Roovers, Seppe Kelchtermans, Kathleen D'Hondt

Department Economy, Science and Innovation – Flemish Government, Koning Albert II-laan 35, 1030 Brussel, Belgium

Introduction

The development of personalised, stratified or precision medicine is an evolution that cannot be stopped. We agreed to use personalised medicine as "**a medical model using 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**" as defined by the Horizon 2020 Advisory Group. This definition was also used in the Council conclusions on personalised medicine for patients (2015/C 421/03), published on 7 December 2015.

The defined in this way, it underlines the importance of the technological developments, which allow to move at much higher pace and towards more profound understanding of what determines health and disease or aging. At the same time the convergence of advanced bio- and nanotechnologies leads to early and accurate diagnosis and to the development of novel treatments, therapies and follow-up. The goals of healthcare are moving from symptom treatment towards ensuring lifelong health by preventive and personalised interventions, hence making personalised medicine or health reality. Bringing personalised medicine within reach of all will induce a transformative change in society, not only by reducing (the length of) chronic diseases, and increasing healthy life years, it will also ensure the sustainability of healthcare systems and boost the development of novel technologies.

Making personalised medicine a reality has been strongly supported by the European Union, starting with a series of workshops almost ten years ago in 2010. Continued support through the seventh and eight (Horizon 2020) framework programmes, the efforts supported several initiatives further paving the way to bring personalised medicine to European citizens.

The Permed project identified important challenges to implement personalised medicine, that may until today still be relevant to fully grasp the opportunities:[1]

- Developing Awareness and Empowerment
- Integrating Big Data and ICT Solutions
- Translating Basic to Clinical Research and Beyond
- Bringing Innovation to the Market
- Shaping Sustainable Healthcare

[1] www.permed2020.eu/_media/PerMed_SRIA.pdf

With the establishment of the International Consortium of Personalised Medicine - ICPerMed - as a follow-up of the Permed project, research as a main driver of personalised medicine should help to realise the following goals:[2]

- Establish Europe as a global leader in personalised medicine research;
- Support the personalised medicine science base through a coordinated approach to research;
- Provide evidence to demonstrate the benefit of personalised medicine to citizens and healthcare systems;
- Pave the way for personalised medicine approaches for citizens.

It was realised that many European regions have strongly research and innovation programmes relevant for the development of personalised medicine and engage in bringing personalised medicine to their citizens. A workshop in May 2017 indicated that the authority levels for issues relating to personalised medicine in regions and countries are fragmented and different among the different regions and in different countries.[3] Addressing the region-specific needs may help to bring personalised medicine more broadly within reach throughout Europe.

To stimulate developments in personalised medicine and support the uptake in all European regions, including sparsely populated and remote regions, the EU awarded a coordination and support action (CSA) on Securing the Adoption of Personalised Health in Regions - SAPHIRe. The project is meant to support the agenda of the ICPerMed, which was formally established in November 2016 at the initiative of the European Commission.

SAPHIRe aims to support regions in Europe to structure the implementation and adoption of personalised medicine in regional healthcare systems by identifying and addressing specific regional gaps and barriers. To reach these goals, SAPHIRe will create a network of regions and their ecosystems, including all stakeholders across the entire value chains. Regions are well placed to bring personal medicine and healthcare closer to the citizen. Pilot projects in and between regions could provide the much-needed evidence for the adoption of personalised medicine in regional as well as national health systems.

A major part of the work will be to identify assets and specific needs and barriers, next to best practices. Getting thorough insight in the (level of) development and implementation of personalised medicine in different regions may also help to identify options to create added value by interregional collaboration. In this respect there will also be alignment with the smart specialisation partnership on personalised medicine (S3P4PM).[4]

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[2] https://ec.europa.eu/research/conferences/2016/permed2016/pdf/towards_ic_permed.pdf#view=fit&pagemode=none

[3] https://ec.europa.eu/research/health/pdf/report_workshop_regions_052017.pdf

[4] http://s3platform.jrc.ec.europa.eu/personalised-medicine

As a final outcome SAPHIRe aims to develop a modular roadmap that can be adapted to the specific regional needs, to support the implementation of personalised medicine.

To reach its goals SAPHIRe will bring regions together in a series of workshops addressing different aspects interfering with the implementation of personalised medicine. For this first workshop regions involved in the S3P4PM partnership were invited together with regions that are members from EIT Health Innostars. In this way we hope to gather a mix of central and densely populated regions, remote and sparsely populated regions, and regions with different innovation status as indicated in the Regional Innovation Scoreboard.[5] The different types of regions are likely to have different needs and different levels of implementing personalised medicine, while personalised medicine ought to be within reach of the broad society in Europe.

The first workshop is meant to provide a platform for regions to discuss their regional-specific barriers and needs interfering with the implementation of personalised medicine and search for ways to overcome these. Addressing pains and gains may be a basis to ensure constructive interregional partnerships.

With this discussion paper we want to provide a basis for discussions during the workshops and give an overview of the regional input. The outcome of discussions in the workshops will be summarised in policy briefs that will be published on the website of the project (www.saphire-eu.eu).

1. *Barriers and needs*

A variety of barriers and needs could be envisioned, and many different initiatives already addressed the barriers interfering with the implementation of personalised medicine. Horgan *et al*. (2014) categorised these barriers in 7 main areas:

- Stakeholder involvement
- Standardisation
- Interoperable infrastructure
- Healthcare system
- Data and research
- Funding
- Policy making.[6]

To a large extend these barriers are similar to the challenges identified in the Permed Strategic Research and Innovation Agenda.[7] The Action Plan of ICPerMed was set up to address these challenges with research as the main driver.

Next to these overarching barriers or challenges for personalised medicine in general, the diverse functioning and authority levels, the different innovation levels and different

[5] http://ec.europa.eu/growth/industry/innovation/facts-figures/regional_en
[6] https://www.permed2020.eu/_media/PHGJ_Barriers_PM_in_Europe.pdf
[7] https://www.permed2020.eu/_media/PerMed_SRIA.pdf

geographic specificities among the different European regions are likely to have different requirements to implement personalised medicine. National and regional health systems differ in several aspects and in the way personalised medicine approaches have been implemented. The analysis of different health systems and identification of best practices will be an inspirational source to broadly implement personalised medicine across European regions, including the sparsely populated or remote areas and regions of different innovation levels.

Regions may provide important assets to addressing these overarching barriers. The discussion during the workshop is meant to get insight in the region-specific needs to fully realise the potential of personalised medicine. With the invitations to the workshop we invited participants to give an appreciation how

- Awareness and empowerment;
- Integration of big data and digital solutions;
- Translating basic research to clinical research;
- Bringing innovation to market;
- Sustainability of healthcare;
- Availability of data;
- Fragmentation of data;
- Privacy issues;
- Impact of GDPR guidelines; and
- Availability & access to funding

interfere with the implementation of personalised medicine, while asking also for other challenges.

2. *Fragmentation of data and the integration of big data and digital solutions*

A first glimpse of regional differences manifested from the small survey. As expected data-related issues are among the most important issues interfering with the implementation of personalised medicine, but while for some regions the amount of data is not so much of a problem, the fragmentation is. It can be expected that regions with centralised health data collection, will not report the amount data as interfering with personalised medicine. Discussions in the workshop should also bring clarity of what the fragmentation is referring to and how the fragmentation could be addressed.

For the development of personalised medicine different aspects related to data are critical. Not only does using personal medicine generate massive amounts of data when identifying the right treatment for the right person, large data sets are an essential requirement to stratify the population, to find unexpected correlations and develop new personalised therapies. The availability and access to large data sets, such as genomics and other omics results, health records, lifestyle information or information from imaging technologies, next to standardisation of data collection and storage are crucial. The integration of data sets covering the different sets of information is expected to create major opportunities for novel applications and better health

outcomes. The data should furthermore be available for re-use and re-analysis upon increased scientific knowledge. Analysing the data using artificial intelligence (AI) can discover previously unknown associations, generate novel hypotheses and drive researchers and resources towards most fruitful directions.[8] Healthcare providers should be ready for the upcoming AI era and embrace the added capabilities that would lead to more efficient and effective care. The integration of decentralised databases in a larger setting is likely to bring added value. Regional barriers are likely to be overcome to achieve this.

In addition, the use of digital monitoring and online or mobile apps that can aid in medical decision making, or the use of video-consults in remote regions.[9] How can we implement these developments within our regions and within our healthcare systems?

3. Privacy issues and the impact of the new GDPR guidelines

Another interesting first result indicates that privacy issues related to health data are reported mostly as very important, while the impact of the GDPR to interfere with the implementation of personalised medicine is estimated lower. The workshop may bring answers of whether the GDPR may perhaps be part of a solution to privacy issues.

Due to the sensitivity of health data, it is of utmost importance to make the data compliant with GDPR regulations and to create trust amongst the data owners and users. Health data is mostly stored in medical settings in the form of electronic health records (EHRs). Throughout Europe and even within countries different types of EHR are being used, and the ownership of health data is questioned. While some regions have the authority level to organise the EHR-system, in others the national level is the authorising level. To bring the same opportunities and levels of healthcare to all European citizens, manners will have to be agreed on how to uniformise these different approaches to health data, so that maximal use of the data can be achieved either for healthcare or for research purposes. Directive 2011/24/EU, that aims to establish a shared European infrastructure to provide access to and allow sharing of EHRs across borders in a secure, efficient and interoperable way is a major step forward. This directive will also allow EU citizens to retrieve his/her medication in a pharmacy located in another EU Member State, thanks to the electronic transfer of their prescription from his/her country of residence to the country of travel. It will furthermore provide patient summaries with background information on important health-related aspects such as allergies, current medication, previous illness, surgeries, etc., making it digitally accessible in case of a medical (emergency) visit in another country.[10] Can a general access to EHR be envisioned regardless of where the person is located, i.e. can a EHR be carried like a passport or identity card? Can personal health data be stored and

[8] Noorbakhsh-Sabet *et al.(*2019), *Artificial Intelligence Transforms the Future of Healthcare*, The American Journal of Medicine

[9] https://www.media.mit.edu/articles/wearable-medical-tech-is-about-to-become-crucial-for-staying-alive/

[10] http://europa.eu/rapid/press-release_IP-18-6808_en.htm

accessed like your personal bank account? And can regions be drivers to establish these developments?

It may be more convincing to citizens to participate and contribute with their health data in research when their health data is collected and stored in regional settings.

4. Translating basic research to clinical research and bringing innovation to market

Interestingly two of the stronger innovating regions reported bringing innovations to market as a major barrier, but report the availability and access to funding as a moderate issue. Most other regions estimated that bringing innovations to market was only a moderate factor towards personalised medicine. As the regional innovation policies differ a lot, better understanding of these, as well as possible best practice examples may help to boost the implementation of personalised medicine.

Not only the development of personalised medicine is expensive, also treatments are, because new applications are no longer one-size-fits-all solutions. Besides this, diagnostics become an essential part of the whole treatment, first to decide on which treatment to use and later to follow up on the outcome of it; this further impacts the healthcare cost. On the other hand of course, ineffective treatments can be avoided, people can be cured faster or diseases can be avoided or postponed. It is clear that the healthcare systems are an essential factor in the implementation of personalised medicine. The reimbursement of novel treatments, diagnostics or preventive measures are determining factors to bring innovations to the market. The healthcare systems in Europe however, are very fragmented, sometimes under regional, sometimes under national authority. Robust evidence base for personalised medicine treatment methods is a major requirement to achieve informed decision making and resource allocation.[11]

Moreover, the use of genomics and developments towards a genomic passport may blur the boundaries between health and disease and support the development of preventive medicine. Healthcare systems should respond to the transformation induced by personalised medicine implementation and also identify the option to include preventive medicine.

Finally, there is a need for standardisation of novel analyses tools, such as microfluidic chips. Different diagnostic toolkits are being developed, that all need specific readout stations. Agreeing on similar chip formats and readout systems would ensure a more broad uptake. There is a general need for standardisation in all aspects of technological advancements, not only as already indicated above for data collection, storage and analysis. Integration of research sites in healthcare settings may maximise synergies and more efficient translation of research into practice.[12]

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[11] Payne K. and Annemans L. (2013), *Reflections on market access for personalised Medicine: Recommendations for Europe*, Value in health

[12] Harvey A. (2012), *The future of technologies for personalised medicine*, New Biotechnology

5. *Availability and access to funding*

ICPerMed suggested to establish a new collaborative funding organisation model with healthcare providers to facilitate investment in disease prevention research and therapy research.[13] They acknowledge that a system of competitive grants to individual researchers is not always the most appropriate way to finance personalised medicine research. It was argued that it may be more beneficial to use a model that recruits and funds groups of top researchers and healthcare providers who agree to work as collaborators in solving problems on disease prevention and therapy. Availability and access to funding was in our preliminary survey not identified as the a major barrier. The discussion in the workshop may address the ICPermed ideas from the viewpoint of regional policy makers and scientists. Can dedicated projects between healthcare providers and scientists create faster solutions and healthcare systems be part of such projects?

6. *Awareness and empowerment*

Awareness and empowerment was in the survey not identified as a too severe factor interfering with the implementation of personalised medicine.

Nevertheless, the empowerment of citizens to support research by providing access to life style and dietary data are important resources for better understanding what determines health and wellbeing and will help to develop novel personalised medicine and preventive approaches. Empowerment of citizens may be a valuable asset, that can be easier achieved in regional settings.

Not only the empowerment of citizens is an essential issue for personalised medicine to become fully integrated, also the awareness of healthcare professionals to turn to the most advanced technologies and ensure that the wealth of health parameters, are used in a uniform decision-making system for diagnoses and treatment. Dedicated skillsets are needed for the implementation of personalised medicine. Even though physicians acknowledge the importance and need for a more personalised approach often the training, support, knowledge, and insight is not following the most advanced developments.[14] Dedicated training programmes for both the biomedical/biotechnological professionals developing the tools/products for PM as well as the healthcare professionals administering the products should support the implementation of PM and ensure the rights skills are present. Curricula may need to be prepared for the jobs of the future. Not only technologies are affecting the education and training landscape, there is also a shift in the traditional doctor-patient relationship. Patient-empowerment and self-management demand also a re-positioning of the healthcare system at large. Education and training programmes may need to take

[13] https://www.icpermed.eu/media/content/ICPerMed_Actionplan_2017_web.pdf

[14] Bonter K (2011), *Personalised medicine in Canada: a survey of adoption and practice in oncology, cardiology, and family medicine*, BMJ open

these changing relationships into account as well and also regions are likely to have a role in this.

7. Future perspectives

Personalised medicine will facilitate a move from solely the treatment of symptoms or diseases to an integrated approach where the individual is the centre and prevention is the goal. To secure the adoption of personalised medicine within regions, SAPHIRe sets out to develop a modular strategic roadmap addressing the specific needs in the different types of European regions. Regional input, feedback and sharing experiences and best practices are valuable and essential assets to discuss in workshops and bring new insights to pave the way towards personalised medicine. Over the coming years, the information from the workshops, discussion groups and surveys will feed into a database to centralise important aspects towards the broad implementation of personalised medicine across all European regions. Alignment will also be made to regional smart specialisation initiatives focussing on personalised medicine. SAPHIRe aims to promote in this way the exchange of ideas and experiences and to promote economic value and access for all citizens to the best possible healthcare.