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CSA PERMED: EUROPE’S COMMITMENT TO PERSONALISED MEDICINE

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Summary: Personalised medicine is one of the most innovative areas in the future of health research. At present, its full potential cannot be developed due to fragmented activities, insufficient communication, and lack of generic solutions in the different areas of personalised medicine; moreover, implementation is a major challenge. The EU-funded Coordination & Support Action PerMed was initiated to step up coordination efforts between key European stakeholders, to allow synergies and avoid duplication or competition, and to provide recommendations to foster the implementation of personalised medicine in transnational research and health systems.

Keywords: Personalised Medicine, Strategic Research and Innovation Agenda (SRIA), Europe, PerMed

Personalised Medicine: present and future

Health care as we know it is radically changing to give way to increasingly more personalised health interventions for citizens and offering more personalised therapies and treatments for patients. Essentially, Personalised Medicine (PM) is an innovative method of treating citizens and patients that utilises research, data and up-to-the-minute technology to provide better diagnostics and follow-up for citizens than is currently the case. Among others, it uses genomic information to discern whether a particular intervention will work for a particular patient and assists clinicians in deciding which treatment will be the most effective. It can also have a huge impact in a preventative sense (see Box 1).

However, we are only at the beginning of the road and many challenges have to be overcome in order to benefit from PM’s full potential. The era of ‘one size fits all’ in medicine is slowly coming to an end. Personalised treatment options are being developed for an array of conditions and some have already entered the market. Treatments for cancer are leading the field, but they are followed closely by treatments in cardiovascular, pulmonary, infectious and psychiatric conditions, among others. Personalised therapies aim to provide “the right treatment to the right patient at the right time”, with early diagnosis, increasing efficacy, decrease in adverse drug reactions, and cost-effective treatments that may result in cost savings, quality of life improvement, and reduction of general morbidity in the population.
Despite being a concept already applied by Hippocrates more than two thousand years ago in Ancient Greece, the advances in the so-called “omic” sciences (genomics, transcriptomics, proteomics, metabolomics, etc.) and in Information and Communication Technologies (ICT) have led to enormous advances in the field of PM in the past two decades. Greater understanding of the molecular basis of disease and all the factors, such as environmental factors influencing disease onset, progression and response to treatment, together with the staggering fall in the costs of gene or genome sequencing and genotyping, plus faster results availability, have resulted in the market entry of over twenty personalised therapeutics; such as Herceptin, the first personalised treatment approved sixteen years ago for HER2+ metastatic breast cancer. The development of many more is underway. However, the scope and approach of PM is not limited to the treatment of diseases only; it is much broader and inclusive, also covering areas such as lifestyle advice, prevention, environmental interventions and even the structure and organisation of hospitals and health systems. Furthermore, the increased interest from physicians, decision makers, regulators and the general public in PM has contributed to its increased application. Figure 1 shows the number of publications on PM in the past 40 years, and their exponential rise particularly in the last ten years. Notwithstanding the great interest, we are still only at the start.

Although the US may have been leading the field in the past, Europe is showing a clear commitment to PM. Reports have been published by the European Commission (EC) supporting the mission of personalisation of health and many politicians and decision makers have expressed their support. For example, in the UK, Germany and France, the national governments have made a strong commitment – implicitly or explicitly – to genomic medicine and the application of PM, mostly in cancer research and treatment. Furthermore, the EC’s new Horizon2020 research grants programme – initiated in January 2014 – will promote research in all aspects of targeted therapies, including ICT to assist decision-making in PM. As the Director General of DG Health and Consumers (DG SANCO), Paola Testori Coggi, puts it “it is essential for Europe to build on our strengths to develop innovations to promote growth and benefit European citizens. Genomics has the potential to be a key sector contributing to this in the future … Advances in PM can bring business development and economic growth to Europe in addition to improved prevention, treatment and care to European citizens.” These objectives form the basis of CSA PerMed, otherwise known as the Coordination & Support Action (CSA) Personalised Medicine 2020 and beyond (see Box 2).

Realising potential benefits

Why should we strive towards the personalisation of health care and promote the four Ps in health (predictive and preventive, personalised and participatory)?

There are potential benefits from applying evidence-based personalised treatments, including:

- improvement of informed medical decisions
- shift from reaction to disease towards prevention and prediction of disease
- targeted therapies with higher probability of success
widely discussed and described in a large number of reports and publications (see next paragraph for examples).

The way forward

PerMed has identified and evaluated the information already available as well as the strategy documents published by key stakeholders, including reports, guidelines and roadmaps on PM. A gaps and needs analysis was performed on 18 relevant reports – from the EC, the European Science Foundation (ESF), the European Alliance for Personalised Medicine (EAPM), the Public Health Genomics European Network (PHGEN), the European Medicines Agency (EMA), the iNNOVAHEALTH Conference under the Cyprus EU Presidency and the European Hospital and Healthcare Federation (HOPE) among others – and over 35 interviews were carried out with relevant stakeholders.

Regarding the mandatory change in mindset in health care delivery:

1. Targeted research in molecular mechanisms and ICT

Targeted research to better understand the molecular mechanisms of disease and all implicated factors, as well as the identification and validation of biomarkers, is essential for the development of further personalised therapeutics. Multidisciplinary research teams, joining the knowledge from a variety of sciences, together with cross-disciplinary and cross-border collaboration in research and in drug development are essential parts of the R&D process of PM. Further developments in data collection, storage, management, sharing, mining, processing and analysis are also imperative. ICTs have not been exploited to their full potential and will surely push forward the individualisation of medicine in all areas (research, translation, diagnosis, treatment decision-making, follow-up, etc.).

2. Adaptive business models, transnational pathways and systematic early dialogue

The current business model for pharmaceutical companies is no longer valid once we move away from the “one size fits all” drugs. Pre-competitive collaboration between companies (pharmaceutical companies and medical device manufacturers, for example), the increase in public-private partnerships and a more flexible and adaptive business model is needed for the development and translation into health care of personalised technologies. Furthermore, systematic early dialogue with regulators and patients at an early phase of development would lead to more efficient drug development and translation processes. Clinical trial designs need to change: Phase III studies with thousands of patients are not possible and adaptive designs with smaller numbers of patients are needed, like the ones already being conducted in cancer that permit the application of personalised treatment options under one protocol. New dynamic and sustainable pathways that lead to timely and effective translation of innovative technologies into health policies and health care are needed, always ensuring high quality, safe and efficient treatments entering the market.

3. Make regulation simple, coherent and predictable

In addition, the regulations that are in place nowadays do not consider the specificities of personalised interventions, including therapeutics. Many of the ones that affect PM are being revised, but remain far from ideal. Especially in Europe – considering the inherited heterogeneity of our Member States – simplified, harmonised, coherent (across directives and regulations) and predictable regulatory procedures are welcomed. Some positive steps forward are the new medical devices directive regulating (for the first time in Europe) in-vitro and companion diagnostics, and the proposed adaptive licensing model from EMA.

In order to expand its leadership role, it is

Box 2: The PerMed consortium

CSA PerMed is a consortium – created by decision makers in Europe, including more than ten ministries and funding bodies – which aims to prepare Europe to be a global leader in the implementation of PM. It differs from other consortia and working groups due to the partners involved and its aim to carry out focussed discussions on concrete research actions, rather than prolonging on-going broad discussions and recommendations (see www.permed2020.eu). Moreover, transparency, openness, collaboration and the avoidance of duplication lie at the core of the CSA PerMed approach. The consortium’s unique features create the potential to develop a strategic research and innovation agenda for Europe (SRIA) and be the starting point for a European Innovation Partnership (EIP) in PM acting across the entire research and innovation chain, bringing together key actors at European, national and regional level.

- risk reduction with fewer adverse reactions to medicines
- timely/early disease interventions
- cost-efficient treatment solutions and general health care cost containment.

For health systems as a whole, potential benefits include early systematic dialogue between the relevant key stakeholders, citizen-centred health care systems, encouragement of patients to be more active in their health management and feel greater ownership in the responsibility of their health, support quality of life, health and wellness, yield a maximum return on health care investment and adjustment to the needs of sub-sectors of the population, among others.

Nevertheless, a great deal still needs to be done to reach these benefits across the entire health care spectrum, and not restrict them to a limited number of conditions. The challenges have been
PerMed’s view that Europe could engage in international efforts to harmonise regulatory aspects.

4. Driving health care systems towards preventive care

When it comes to health care systems, a general change in mind-set in health care delivery and provision is needed. From a coordinated reimbursement process for drugs and diagnostics, new financing strategies, new structures and models at the provider level, updated health care professional training and a change in attitudes, a shift towards preventive care, towards new cost assumption models, changes in patient behaviour and an increased interest and literacy from citizens in general are needed. The social consequences of the implementation of PM have not been fully studied, and there are many ethical challenges that lay ahead, which is why the principles of “Ethical, Legal and Social Implications (ELSI) are essential and need to be further explored by research and applied by all stakeholders.

Conclusion

Even though PM may be one of the most innovative areas in the future of health research, the full potential for patients, citizens and the economy in Europe currently cannot be realised due to the inherited fragmentation between European Member States, inadequate communication and lack of common vision on the solutions that are needed. Appropriate governance levels are required to solve these challenges.

PerMed aims to provide concrete recommendations and to take a big step forward towards PM for all, without forgetting that the ultimate goal is to bring the right health intervention to the right patient at the right time, to avoid as many adverse reactions as possible during treatment, to make it affordable for health care systems and to ensure equality in access to personalised innovations. As long as the interests of citizens drive work towards this common mission, Europe can become a leader in PM, with the potential to also create business and economic growth and, most importantly, give patients access to safe, highly efficient and targeted treatments in a timely and cost-efficient manner.

References

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