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Personalised Medicine Conference 2016

Report

Research and Innovation

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Personalised Medicine Conference 2016

Report

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EXECUTIVE SUMMARY

The goal of personalised medicine is to put the citizen at the centre of healthcare. It is a new paradigm in medicine based on the smart use of technology, coupled with greater participation by patients in the management of their own health, to help prevent disease and promote healthy living. When diseases can be prevented and not only treated, the cost of healthcare will come down, creating a virtuous circle for health policy. The Personalised Medicine conference addressed the broader policy perspective and challenges by showcasing integrated healthcare models in Member States and business approaches which involve patients more directly in their healthcare. The meeting was organised around five challenges.

Challenge 1. Developing awareness and empowerment.

Two cancer survivors outlined what today's patients want from policymakers. This can be summarised in one sentence: to be involved in decision-making affecting their own healthcare. This means involving patients in the formulation of treatment guidelines and protocols, the design of clinical trials and medicine reimbursement. Medicine will increasingly be powered by patient data. Therefore it is critical that systems are in place for obtaining patient consent, and patient participation in decision-making about the use of those data.

Challenge 2. Integrating big data and ICT solutions.

Making technology work *for* patients. This concept was illustrated by IBM's Watson Health unit which has developed artificial intelligence with algorithms that analyse databases to help doctors with diagnosis. Furthermore it was explained how Scotland uses information technology to predict and manage disease on a population level within the Scottish National Health Service. Another example was presented from Estonia, which has organised its entire health system around electronic registries. These enable patients, doctors and other healthcare professionals to conduct transactions such as ordering prescriptions, while also supporting basic research. A challenge for policymakers is to ensure that the system for obtaining patient consent is robust and the purpose for which it will be used is transparent. A related issue is managing new data sources effectively. Some doubt was aired about whether practitioners currently have all the analytical tools and standards in place to do this. It was suggested that EU Member States establish their own institutes for biomedical data and informatics which could then be joined up at EU level. The challenge is to standardise and validate these systems without losing sight of the purpose for which they were intended – to improve patient care.

Challenge 3. Translating basic to clinical research and beyond.

The discussion about information technology was taken further by illustrating how new sequencing technologies can be used to identify gene mutations, which in turn become targets for developing novel therapies. Genomic and phenotypic data can be combined with epidemiological data to design disease prevention campaigns. The North Karelia Project in Finland succeeded in reducing mortality rates from chronic heart disease by means of a sustained campaign. Good results were also achieved in the clinical arena by researchers in Belgium by identifying a mutation present in a subgroup of cancer patients and repositioning a marketed drug for those patients. In stratified clinical trials, patients are selected based on the likelihood of their response to a new treatment. All three examples illustrate the need for practitioners to work with well-defined targets, monitor the results carefully, and be prepared for the unexpected. A challenge for policymakers is to have a system in place for validating biomarkers for use in trials and public health campaigns. Currently, different companies produce different biomarkers for the same indications. These efforts need to be consolidated.

Challenge 4. Bringing innovation to the market.

This discussion focused on incentives for bringing personalised medicine to the market. Each personalised medicine approach or drug will be developed for a relatively small patient population. Companies need incentives to undertake this work, which will not be as remunerative as developing drugs for a large market. Options discussed included a risk-sharing agreement under which the public authority would guarantee a market share in exchange for an agreement to undertake the risk of drug development. It was also suggested that in order to ease market entry for new products, gaps in the regulatory system could be closed. This would require bringing health technology officials into talks with pharmaceutical regulators, so that prospective new drugs and approaches were given the best chance of market access, as is already practised by the European

Medicines Agency. Finally, speakers referred to certain aspects of EU policy on orphan drug development which might be adapted to personalised medicine products. The orphan drug policy is seen as a huge success, as it combines industry incentives with clearly defined patient populations and encourages patient participation in the drug development process. Any adaptation of this policy to personalised medicine would require guarantees to contain prices and avoid stifling innovation.

Challenge 5. Shaping sustainable healthcare.

To be sustainable, any strategy for personalised medicine needs to enjoy broad support from the population. This starts with having sound policies on informed consent and the use of personal data. It continues with the building of electronic patient records, registries and biobanks, all of which need to be integrated into a system that has practical benefits for people. The benefits include information on disease prevention and treatment and the importance of a healthy lifestyle. If the system is synchronised and enjoys public support, there are massive opportunities for improving public health and bringing the cost of healthcare down.

INTRODUCTION

The following report is a summary of the discussions and presentations which took place at the Personalised Medicine Conference 2016 on 1-2 June in Brussels. The presentations and a video recording of the proceedings can be viewed online at: www.ec.europa.eu/permed2016. The views and opinions expressed in this report are not necessarily those of the European Commission. The summary of the presentations and interventions of the speakers should be checked against actual delivery.

Personalised medicine is an approach to healthcare that puts the citizen in the centre. By developing tailor-made diagnostic, treatment and prevention strategies, patients receive therapies that specifically work for them. It also allows people to participate in the management of their own health by having access to information about the prevention and treatment of disease. There is no universally accepted definition of personalised medicine and the concept is evolving with the advance of technology. A definition which is gaining acceptance in Europe was however presented (see keynote session).

Already in 2011, a European Commission conference on the subject highlighted the role of molecular diagnostics in helping healthcare professionals identify which patients were most likely to respond to specific interventions. New diagnostic technology was making it possible to match patients with the most appropriate treatments. Since then diagnostics have become more sophisticated, and a revolution in information technology has made it possible for researchers to collect, store and analyse ever-larger quantities of data that are relevant to patient care.

On 1-2 June 2016, the European Commission held a second conference on personalised medicine, this time to discuss a broader policy perspective. This is to create a new ecosystem in the EU that would bring together research institutions, patients, healthcare practitioners and governments to use today's vast data resources to foster the well-being of its citizens by preventing disease, or when disease does strike, to manage it better. It is clear that personalised medicine requires a new mindset. Putting the patient at the center of healthcare will require innovation in the way medicines are developed and healthcare systems are structured to deliver care. Under this new paradigm, the patient ceases to be the subject of research or treatment and instead becomes an active partner. This will require a big adjustment amongst all participants in the healthcare system. But the potential rewards can be significant: better healthcare at more affordable prices.

The conference was organised around five challenges:

- 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.

These challenges have been developed and discussed in the report *Shaping Europe's Vision for Personalised Medicine* of the EU funded project PerMed (www.permed2020.eu) and published in June 2015. Recommendations from this report, together with conclusions from the conference, will form the basis of a new initiative to be called the International Consortium for Personalised Medicine or IC PerMed[1].

Together the member organisations of IC PerMed, which currently include more than 20 funding bodies from the EU member states and beyond, will work to:

- 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.

[1]See the information sheet "Towards an International Consortium for personal Medicine" on: https://ec.europa.eu/research/conferences/2016/permed2016/pdf/towards_ic_permed.pdf

Members of the consortium will be public and private not-for profit health research funding and policymaking organisations. They will use their own funding rules and policy processes to contribute to the overall goals of the consortium. IC PerMed will be formally launched in November 2016. Until then, IC PerMed is being run on an ad-interim basis by an executive committee and groups representing the five challenges. The executive committee is currently chaired by the Commission.

IC PerMed will develop a roadmap with recommendations for action organised around the five challenges mentioned above. The first version of the roadmap will be published at the end of 2016. The document will be updated at regular intervals thereafter. The IC PerMed members will work together to implement the actions.

The work around the five challenges is led by Challenge Facilitators from the IC PerMed member organisations. These facilitators will oversee the implementation of the roadmap. They also helped organise the conference and chaired the thematic sessions. The table below shows the names and organisations of the Challenge Facilitators.

Challenge	Challenge Facilitators
Developing awareness and empowerment	Maria Judith Molnar, Semmelweis University, on behalf of the Health Ministry, Hungary
Integrating Big Data and ICT solutions	Wolfgang Ballensiefen, Project Management Agency DLR-PT, on behalf of the Education and Research Ministry, Germany
Translating basic to clinical research and beyond	Hemma Bauer, Science, Research and Economy Ministry, Austria Natalia Martin & Daria Julkowska, National Research Agency, France
Bringing innovation to the market	Peter Høngaard Andersen, Innovation Fund Denmark
Shaping sustainable healthcare	Gaetano Guglielmi, Health Ministry, Italy

KEYNOTE SESSION

Carlos Moedas, EU Commissioner for Research, Science and Innovation, opened the meeting with a video message saying that personalised medicine is an area where the Commission can make a real difference. He outlined how this approach puts the patient front and centre and has started to gain traction with targeted therapies in areas such as cancer, while acknowledging that there are challenges ahead, which is why the Commission wants to set up IC PerMed. As highlighted by the Commissioner, the focus of this consortium will be to make Europe a global leader in personalised medicine, define the research challenges and develop the science and drive innovation.

Ruxandra Draghia-Akli, Director of the Health Directorate, Directorate-General for Research and Innovation, gave an overview of the subject. There is no universally accepted definition of personalised medicine. While there are other ways to describe patient-centric healthcare, such as stratified medicine and precision medicine, the Commission has elected to use the term personalised medicine. The definition, which is the basis for IC PerMed is the same as that used in the Council of the European Union conclusions on personalised medicine for patients (2015/C 421/03) and the Horizon 2020 work programme for Societal Challenge 1: Health, demographic change and well-being[2].

According to this definition, personalised medicine "…refers to a medical model using characterisation of individuals' phenotypes and genotypes (eg 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."

The idea is to use multiple information sources to make healthcare smarter with a greater emphasis on disease prevention and prediction. This means shifting the focus from treating disease to managing health. The Commission was an early mover in the field, already in 2011 it looked at the role of the 'omics' disciplines in helping understand the causes of disease. The goal now is to support the personalised medicine science base through a coordinated approach to research and innovation, together with the IC PerMed members and the involved member states, and to show the benefits of this approach to citizens and healthcare systems.

IC PerMed is based on a series of challenges, which in turn will be translated into a roadmap, expected to be available in the fourth quarter of 2016, for implementing policy at an EU, national and regional level.

Robert-Jan Smits, Director-General for Research and Innovation, said that personalised medicine goes beyond the scope of pharmaceuticals to include other industries. It promises to make healthcare smarter and proactive and it is in line with the Commission's priorities of supporting cutting edge research, driving innovation and creating new markets and jobs. The launch of IC PerMed is being undertaken with the cooperation of research funding and policy making organisations in EU member states and beyond. It has been inspired by other international consortia, for example in the fields of epigenetics and rare diseases: the International Rare Diseases Research Consortium (IRDiRC) and the International Human Epigenome Consortium (IHEC) (www.irdirc.org and http://ihec-epigenomes.org) respectively. IC PerMed is a coordinated approach to research and innovation. It will rely on the ability of participants to integrate data from multiple sources and use this information to improve health without affecting patient confidentiality.

Xavier Prats Monné, Director-General for Health and Food Safety, spoke about the future of EU healthcare systems. He noted that inequalities still exist within healthcare systems at a national level. Personalised medicine is an opportunity to look at new ways of delivering healthcare, assessing healthcare technologies and monitoring regulatory systems to make sure that they are keeping up with innovation. It is an approach to healthcare that presents an opportunity to bring people together to work on big issues of common interest.

Lowri Evans, Director-General for the Internal Market, Industry, Entrepreneurship and SMEs, said that personalised medicine is a route towards improving the competitiveness of European industry. There are opportunities for industry to develop new business models based on the widespread use of digital technologies. Personalised medicine also goes hand-in-hand with the development of

[2] The Council conclusions on personalised medicine for patients can be consulted on the web page: http://eur-lex.europa.eu/legalcontent/ EN/TXT/?uri=uriserv:OJ.C_.2015.421.01.0002.01.ENG&toc=OJ:C:2015:421:FULL

companion diagnostics. The new Regulation on IVD medical devices is expected to strengthen oversight of companion diagnostics by increasing transparency and the powers of notified bodies, which certify these products. While the regulatory aspects of some of the new technologies are being addressed, there remains the issue of the cost of personalised medicine. While healthcare resource allocation takes place at a national level, there is an opportunity for IC PerMed to bring all the actors together to support them with the best available information.

Roberto Viola, Director-General for Communications Networks, Content and Technology, addressed the issue of data. Without data, there can be no personalisation of medicine. Yet Europe could be stronger when it comes to super-computing and data storage. The EU market is the largest in the world, and therefore should be fully equipped to navigate the digital economy. For example, computing power needs to be increased, with the possibility of creating a European science cloud. The Directorate-General for Communications Networks, Content and Technology plays various roles in the personalised medicine initiative; considering activities in e-Health, Big data and High Performance Computing. The regulatory aspects are equally important: such as data flows, cybersecurity and data exchanges.

Paulo Lisboa, Professor and Head of Department of Applied Mathematics at the John Moores University in Liverpool, UK talked about the challenge of shifting from a disease-oriented healthcare system to one focused on well-being. Using breast cancer as an example, he said molecular analysis has shown that there is not one, but several types of the disease. In addition there are similarities between breast and prostate cancers. The pharmaceutical industry is now developing medicines against these targets. Yet will these new treatments help a woman with cancer who also lives in a deprived area and may also be suffering from obesity and diabetes? Personalised medicine may be a way of closing the gap between clinical medicine and the other aspects of real life that affect human health. These differences can be captured in data, but patients must consent to provide this data. The level of consent required for advancing personalised medicine is not trivial. Another question is whether it will be possible to produce a better quality of care at a reduced cost. This will require a shift from a system that reacts to disease to one that seeks to prevent disease. To accomplish this, patients will need to agree to manage their own health. Patient-reported outcome statistics will make it possible to establish which interventions are necessary and which are not.

Anders Olauson, Honorary President of the European Patients' Forum, ended the session with a call for patient empowerment. This entails giving patients access to information that will enable them to work with doctors in the management of their own healthcare. Patient input will also be necessary in assessing the value of new therapies.

CHALLENGE 1: DEVELOPING AWARENESS AND EMPOWERMENT

This session was devoted to a discussion of the patient's view of personalised medicine. Maria Judit Molnar, Professor at Semmelweis University in Hungary and Challenge Facilitator for IC PerMed Challenge 1, opened the meeting with a summary of the key aspects. Personalised medicine puts the patient at the centre of healthcare decision-making. She illustrated this with an example of a woman whose aunt had a gene mutation which was predictive of cancer. After genetic counselling, the woman asked to be tested and discovered that she too was positive and had a risk of developing cancer. She began to educate herself with books and on the internet. Knowledge about the genome and patient literacy empowered her. This points to a new model for care where the patient is engaged in researching aspects of his or her own health.

Diagnosed with lymphoma in 2005, Peter Kapitein is a founder of the patient advocacy group Inspire2Live. Based in the Netherlands, Inspire2Live has about 34 members who are living with cancer. They meet with clinicians, scientists and business people to identify parts of the cancer healthcare system that could be improved. The group supports a fundraising event each year where cyclists bike up and down Alpe d'Huez in the French Alps six times in one day. Mr Kapitein had four pieces of advice for the meeting. They are: engage people with different perspectives; search for the root cause of a problem; think on a big scale, and remain independent.

Rudi Westendorp of the Center for Healthy Aging, University of Copenhagen, spoke about the relevance of personalised medicine to ageing. Prof Westendorp is author of the book, Growing Older Without Feeling Old: on Vitality and Ageing. He said that elderly citizens are concerned about disease but also about the quality of their lives. This will require a new focus on research to

promote healthy ageing. However the health and social care services for elderly citizens are fragmented. One tool for "helping people help themselves" is a data cooperative called MIDATA. Developed by Switzerland-based researchers, MIDATA enables citizens to securely store and manage personal healthcare data. Members of the cooperative can decide to share their data with doctors or participate in medical research. Another collaboration, dating from 2008, is the large healthcare initiative EIT Health which addresses healthy living and active ageing. EIT Health gets support from the European Institute of Innovation and Technology (EIT) which is an integral part of Horizon 2020. EIT Health is a consortium of businesses, research centres and universities which work together to promote active ageing and healthy living. This is one initiative to bring healthcare companies together to help the end-user, Prof Westendorp said.

Jan Geissler, Director of the European Patients' Academy on Therapeutic Innovation, told the meeting that patients need to be involved early in the clinical development of new medicines. A cancer patient for 15 years, Mr Geissler has many advocacy roles including as a co-founder of a global network of leukaemia patients. He said patients should be involved in the design of clinical studies, not just the final stages of these trials. The HIV advocates also helped set research priorities and the design of protocols for new trials. The European Medicines Agency has set an example by involving patients in some of their committees. But more could be done in the EU member states, he said.

Panos Kanavos of the London School of Economics said that more needs to be done to involve patients in health technology assessments (HTA). HTA analyses look at the scientific rationale and evidence but they don't have appropriate metrics for making social value judgements. These social values include what interventions are appropriate for patients at the end of their lives. Only patients and their carers know exactly how a disease impacts them personally and how specific treatments can affect their life quality. He cited a survey of patients that sought to discover whether there were important aspects of their illnesses which had not been captured by a research tool called EQ-5D-5L. Fifty-one percent of respondents said 'yes.' Some of the problems that weren't being captured related to family relationships and finance. Dr Kanavos said current HTA gives priority to general population values such as patient mobility. But in fact patients themselves may be more concerned about pain, anxiety and depression than mobility. Health economists need to revisit the methods for capturing patient values in HTA.

Panel discussion

In the discussion that followed, speakers said that patients have too long been viewed as subjects of research rather than active partners. György Németh of Gedeon Richter Plc captured the mood when he said that if patient engagement were a drug it would be the "blockbuster of the century." This engagement starts with defining the research question and is followed by the development of a new drug. Peter Kapitein asked why, for instance, patients are not consulted about which diseases to investigate. Maired O'Driscoll of the Health Research Board in Ireland said there is a great demand for information about how the health and research systems work. How to engage patients in this process is an important issue. Jan Geissler said the European Medicines Agency does a great job involving patients in some of its discussions. This example could be followed by the national agencies. Furthermore, once a product is on the market, patients need to be involved in reporting outcomes from their treatments. Rudi Westendorp said that the benchmarks used by public authorities to reimburse medicines may need to be revisited to take more account of patient needs.

The following additional points were made:

- We don't yet know how to optimise consultations between patients and their doctors.
- There needs to be more shared decision-making between patients and their doctors;
- Treatment guidelines and protocols are physician-oriented; they need to be more patient-oriented;
- Value-based healthcare has yet to be achieved; there are costs borne directly by healthcare systems. But still to be measured are the indirect costs to families;
- Information obtained from patients about their treatments – patient- reported outcomes – is valuable. It can be used as a basis for discussions between the patient and his/her doctor as well as a framework for discussions with future patients

CHALLENGE 2:
INTEGRATING BIG DATA AND ICT SOLUTIONS

Introducing this session, Wolfgang Ballensiefen, senior scientific officer at the DLR Project Management Agency in Germany and Challenge Facilitator for IC PerMed Challenge 2, said that recommendations from the report Shaping Europe's Vision for Personalised Medicine need to be brought to the next level. Big Data will be the backbone of future research. The healthcare community will need tools that can store this data, which includes clinical information as well as data on lifestyles. Furthermore, research into data collection, storage, harmonisation and security is needed, as well as strategies to make sense of this data. An example of how this can be done is the field of weather forecasting. For example, some cities already use Big Data to predict future weather patterns. Similarly in personalised medicine, both hardware and software approaches can aid research and help optimise health systems. In parallel, solutions need to be found for managing access to data, and data ownership and privacy. This will require research into the ethical, legal and social dimensions and new innovative approaches.

Michal Rosen-Zvi, Director of IBM Research Healthcare Informatics in Haifa, Israel, explained how data is changing the profile of medicine. Two examples are advances that have been made in the treatment of AIDS (acquired immune deficiency syndrome) and cancer. Once a fatal disease, AIDS is now a chronic illness that can be managed with drugs. These drugs have been developed from a greater scientific understanding of the proteins that make up the human immunodeficiency virus (HIV). The protease and reverse transcriptase proteins include more than 300 different amino acids which are relevant to the design of new therapies. This knowledge was gained from data on the structure of the HIV genome. Taking this one step further, physicians have been able to extract samples of the virus from a patient's blood and test these samples against all possible drugs. This enables them to recommend the optimal treatment for patients. The procedure has been computerised thanks to the work of EuResist, an international research project that received funding from the European Commission under the Sixth Framework Programme. EuResist assembled data from 10 European centers covering the medical records of 65,000 patients over a period of 20 years. This was integrated with information about the structure of the virus and licensed HIV therapies. EuResist is now a non-profit organisation managed by five research partners (www.euresist.org).

Cancer is also moving from a fatal to a chronic disease, at least for some types of cancer. But the challenges in developing treatments are different from those of HIV. Genomic information is important but so is data on a patient's diet and lifestyle and on the environment. Dr Rosen-Zvi said cognitive computing comes into play in cancer, particularly in radiology. The company's Watson Health unit has developed artificial intelligence, together with algorithms, that can read the content of databases and sift through medical images (eg mamographies, ultrasound or magnetic resonance imaging), to help doctors find information for a diagnosis. To advance this business, IBM recently acquired a medical imaging company, a population health management company, an insurance business and a healthcare analytics company. The company has also put in place partnerships with Medtronics to develop technology to give people with diabetes an early warning of hypoglycaemia.

Ernst Hafen, head of the Institute of Molecular Systems Biology at ETH Zurich, described a different business model for collecting and analysing health data. This is MIDATA (previously mentioned by Rudi Westendorp in the session on patient empowerment). MIDATA is a not-for profit cooperative consisting of data banks that are owned by members who also contribute the data. All of the data is encrypted and only members possess a key. The technology platform has been developed by software engineers at ETH and Berner Fachhochschule BFH and is accessible via third-party Apps. Patients can access their own data or share their data with third parties. But they take the decision to share it. Any revenue generated by the secondary use of the member-controlled data is invested in products and services that benefit the members and society-at-large. In a reversal of current practice, patients can themselves signal their interest in participating in a clinical trial rather than waiting to be recruited by a contract research organisation. Whereas MIDATA is the name given to the national data cooperatives, MIDATA.coop is the federation of national cooperatives. The federation has a common IT structure and a common data exchange platform, similar to SWIFT. which is the cooperative that provides secure messaging services for the financial industry. Dr Hafen said the cooperative approach gives the citizen control over data collection and use, thus overcoming the distrust many people have with global data companies.

An example of how personalised medicine can be applied on a national level was given by Andrew Morris, director of the Usher Institute of Population Health Sciences and Informatics at the University of Edinburgh. Dr Morris is also the chief scientist for health in Scotland. Using case studies, Dr Morris explained how Scotland employs information technology to predict and manage

disease within the Scottish National Health Service. With a budget of €15.6 billion, the NHS is the single-payer healthcare provider for the five million people living in Scotland. The service consists of 38 hospitals and 1,020 general practices and is governed by 14 territorial boards. Every patient in the system has a unique patient identifier number which is included in a centrally maintained registry. Citizens have electronic patient records that are protected by special security software. The patient records can be linked to other data sources for research purposes, for example, to discover the prevalence of a disease or evaluate the effectiveness of a public health policy. An example of the latter is a survey conducted of the population to determine the effect of legislation banning smoking. The results showed that childhood asthma increased by 5.2% per annum before the ban. After the ban, the disease decreased by 18.2% per annum. In another study, Scottish researchers showed that regulatory concerns about adverse events related to varenicline, a smoking cessation product, were unfounded. And in a third example, they used a genome-wide association study to show that variations in the gene ATM can alter the glycaemic response to metformin, a diabetes drug, in patients with type 2 diabetes.

Dr Morris said the future of personalised medicine depends on being able to harness digital technology. But there are still issues that need to be resolved about governance, data quality, data maturity, standardisation, and trust. This will require data providers and users to agree on standards, clarify responsibility for the flow and control of data and be more transparent about their activities. In the UK, the Farr Institute of Health Informatics Research is setting an example by linking electronic health data with other forms of research to build capacity in health informatics research. The Institute is partly funded by the Medical Research Council. At EU level there are also opportunities to develop health data science. Could the EU lead the way? Dr Morris asked.

Another example of a data cooperative is the Innit Foundation, located in Amsterdam, the Netherlands. Mitzi László, co-founder, explained the concept. The foundation is a not-for-profit organisation that stores encrypted personal data. Connected to the foundation is a commercial company which has links with other commercial entities. Users can store their data in the foundation. If they wish to do so, they can offer their health data for medical research. A system of double encryption protects this information. At the same time users can release data through the company for commercial purposes. In the latter case, the user makes money on the transaction rather than a global company like Google or Facebook, Ms László said.

Janet Thornton, senior scientist and Director Emeritus at the European Bioinformatics Institute (EBI), European Molecular Biology Laboratory (EMBL) in Hinxton, UK, discussed the challenges of interpreting genomic data. Getting this right, will have a direct impact on the uptake of personalised medicine. The human genome consists of three billion base pairs of DNA, of which there are four million variants. These variants are the source of many diseases. Deciphering the variants can be difficult because they are many types. They include single nucleotide polymorphisms, copy number variants, splice site variants, variants in promotors and variants that affect signalling. The variants in turn can have an impact on proteins, protein structure and molecular pathways. Interpreting the data however can be difficult. Could personalised medicine help? Prof Thornton said that developing global public data resources, to identify actionable variants, is a start. Large resources already exist, but they need to be fully public and operate according to global standards. This is already happening through the Global Alliance for Genomics and Health (http://genomicsandhealth.org), and Elixir. The Global Alliance is a group of more than 400 institutions working to create interoperable technical standards for managing and sharing genomic and clinical data. Elixir is a European infrastructure for life science information.

To complete the infrastructure, each country in the EU could establish an Institute for Biomedical Data and Informatics. These institutes would look after personal genomics data. In the larger nations, the institutes would probably be networks. The EU could then launch a project to join up the institutes. Such an effort would build on, and interact closely with, Elixir as well as BBMRI (Biobanking and BioMolecular Resources Research Infrastructure) which is another research infrastructure project. Prof Thornton said training would be required for a new cadre of clinical scientists who are experts in genomic medicine, and in data handling and interpretation. Training would also be required for hospital doctors, nurses and other carers.

Jaak Vilo, Professor of Bioinformatics at the University of Tartu in Estonia, described how information technology can enable personalised medicine when it is integrated into a single infrastructure. Each citizen in Estonia has a unique identification (ID) code, an ID card and a smart card. The Estonian IT architecture includes registries for population, health and vehicles as well as registries for document management, banking and telecommunications. Citizens can gain access to the registries through portals, which communicate with the registries after passing through security software. In 2015, Estonia's 1.3 million citizens directed more than 500 million queries to the registries. The health subset of the IT architecture contains a prescription centre as well as registries for hospitals, family doctors and pharmacies. Nearly all, or 99%, of prescriptions, are obtained electronically, Prof Vilo said.

Besides handling millions of transactions, the system also supports research. For example, the records show doctor visits and tests, and they can show different diagnoses. A retrospective analysis can then find out how much each diagnosis has cost the health system. With people willing to donate information and samples to the biobank, researchers are now performing a variety of analyses including whole genome and exome sequencing, RNA sequencing and measuring telomere length. This information can then be used to determine the risk factors for disease among members of the population. Prof Vilo concluded that personalised medicine needs to be supported by analyses that are derived from electronic health data as well as good genetic databases. The databases should store annotated genetic variants and validated predictive models of disease that can be acted upon.

Panel discussion

The discussion focused on two different models for building databases with personal healthcare information: the commercial model as illustrated by IBM and the co-operative model as represented by MIDATA and the Innit Foundation. The main issue was understanding how a patient's identity is protected under each model and how access to this data is managed. Members of the audience wanted to know whether a person who has donated information to a databank can reverse this decision and get the data back if his or her circumstances change. There were also questions about who gives consent on behalf of children. During the discussion it was noted that consent can sometimes be indirect. Dr Rosen-Zvi said that IBM has acquired other companies in order to get access to data. It didn't buy patient data but it bought companies that have ethical agreements with these patients. Dr Morris said that regardless of the business model, the guiding principle should be transparency. The manager of a database must be fully transparent with the donor about the uses to which the database will be put. Scotland distributes leaflets which explain how it plans to use the healthcare information that it collects.

CHALLENGE 3: TRANSLATING BASIC TO CLINICAL RESEARCH AND BEYOND

This session was introduced by Natalia Martin, of the French National Research Agency (ANR) as the Challenge Facilitator for IC PerMed Challenge 3, serving in that function together with Daria Julkowska, of the ANR, and Hemma Bauer of the Austrian Ministry of Science, Research and Economy.

The session began with a keynote address by Nicholas Katsanis, Director for Human Disease Modelling at the Duke University School of Medicine in the US. Dr Katsanis discussed the challenge of interpreting genetic variations accurately. Citing a study involving the genetic disorder Kabuki syndrome, he explained how scientists used whole-exome sequencing to identify a mutation on a gene called RAP1A in a patient with Kabuki syndrome and another mutation in the gene RAP1B in a second individual with Kabuki syndrome-like features. The scientists constructed a disease model using zebrafish and were able to describe the genetic and functional interactions between the genes. This revealed that the pathophysiology of Kabuki syndrome overlapped with that for RASopathies. RASopathies is a term that describes another type of developmental syndrome. As a consequence, a pathway was discovered for potential new therapies. Dr Katsanis said the experience illustrated the importance of strong genetics and biochemistry and the willingness of scientists to collaborate. He cautioned however against an over-reliance on information technology. Scientists still need time to work out a solution to problems and "give each other the opportunity for serendipity."

Markus Perola, research professor at the National Institute for Health and Welfare in Helsinki, Finland, explained how an important public health problem can be solved through a community-based intervention. The example is the North Karelia Project, a public health programme that sought to address high rates of cardiovascular disease. In the early 1970s the Karelian region in Finland had the highest cardiovascular mortality rates in the world. To tackle the problem the health service, along with partners, set out to reduce the risk factors for disease by encouraging people to stop smoking and reduce the amount of saturated fat in their diets. The project started in 1972 and surveys conducted over subsequent years showed a high rate of compliance. Mortality rates from chronic heart disease showed a significant decline. Dr Perola attributed this success to restricted, well-defined targets, good monitoring of immediate targets, working closely with the community and the media and support from the World Health Organization. What are the lessons

for the future? Family history is still an important diagnostic tool and can be more informative than many genome-based studies. Despite the large amount of data generated from these studies, only a small proportion of the phenotypic variation among individuals was explained. This is an area that merits attention. Meanwhile further studies are needed to explain how practitioners can predict disease progression, or patient response to specific treatments, on the basis of gene variants.

Sabine Tejpar, professor at University Hospital Leuven in Belgium, explained why doing retrospective analyses of trials is important in advancing personalised medicine. To illustrate, she gave the history of cetuximab, a monoclonal antibody therapy approved by the US Food and Drug Administration for advanced colorectal cancer in 2004 (the same year, the European Commission granted it market authorisation under a different name in the EU). The antibody targets cancer cells expressing the epidermal growth factor receptor (EGFR). While the binding of cetuximab to EGFR stopped cancer growth in many patients, it soon became apparent that it did not work for everyone. As a result scientists undertook retrospective analyses to find out why. Their research identified a gene mutation that was present in some patients but not in others. As discussed in several academic papers, patients with a mutation in the K-ras gene resisted the therapy, while those with a normal, or wild-type gene, did not. In 2012 the FDA approved a new indication for cetuximab (followed by an approval by the European Commission in 2013), this time for patients with the K-ras wild-type gene. They also authorised a diagnostic to accompany the drug that can identify patients with the correct genetic profile.

Dr Tejpar said the reanalyses took years to complete, and points to a second issue. This is the need for validated biomarkers to predict disease. Different companies are producing different biomarkers for the same treatments, but these efforts need to be consolidated. As a starting point, neutrally-held biobanks (not owned by companies) should be a standard feature of clinical trials. In this way, retrospective analyses would be easier.

David Jones, professor of liver immunology at Newcastle University in the UK talked about stratification in clinical trials. Professor Jones is also a principle investigator in the UK-PBC Research Consortium, a group that is researching the causes and possible treatments for chronic liver disease (primary biliary cirrhosis or PBC). The only licensed therapy for the disease is ursodeoxycholic acid (UDCA). This drug slows the progression of the disease, but nearly one-third of patients don't respond well, or at all, to the drug. This raises the question of what approach researchers should take to find a better therapy. Professor Jones said that there are significant challenges in designing a clinical trial for stratified therapy of PBC, including lack of relevant biomarkers and the difficulty in performing a histology-based trial.

Panel discussion

Panelists agreed that there is far more scientific collaboration now than five years ago when the first European Commission conference on personalised medicine took place. What is less advanced is the link between fundamental research and clinical research. "The biology is moving faster than drug development and clinical trials," said Fabien Calvo of Cancer Core Europe. For example in liver cancer, researchers have identified at least seven different pathways with up to 30 different genes, as well as a virus, some of which could potentially be treated with new drugs. But there is not enough clinical data to support these hypotheses. This leads to the question of whether the biological samples in biobanks across Europe are adequate to support a large-scale personalised medicine initiative. Some panalists said that sample sizes are too small, and not sufficiently standardised. Jan-Eric Litton said his organisation, Biobanking and Biomolecular Resources Research Infrastructure (BBMRI), is working towards making biobanks more fit for purpose. Better samples mean better biomarkers, he said. These can then be used to design better clinical trials. With regard to Europe's clinical trial infrastructure to support personalised medicine, Jacques Beckmann of the Swiss Institute of Bioinformatics, said the infrastructure was "terrific." However Christine Mayer-Nicolai of Merck KGaA said there was a lack of infrastructure to recruit small numbers of patients across countries, which is what personalised medicine will require.

The following additional points were made:

- Clinical data, including the descriptions of disease, need to be standardised;
- The backbone of personalised medicine is the patient sample. Biobanks, which contain these samples, need to be fit for purpose and exhibit the highest quality;
- Patient samples are important, but so is phenotyping. Phenotypic data needs to be collected in a well-defined manner;
- A European research infrastructure is important, but there also need to be communication efforts directed at the local research community;

- There is a need to develop more biomarkers from proteins.

CHALLENGE 4:
BRINGING INNOVATION TO THE MARKET

Peter Høngaard Andersen, chief executive of the Innovation Fund in Denmark and Challenge Facilitator for IC PerMed Challenge 4, introduced the session by pointing out that some of the most successful medicines are the product of an ecosystem, rather than the efforts of a single company. He used Novo Nordisk A/S's Victoza, a non-insulin medicine for diabetes, as an example. Victoza was approved for marketing in the EU in 2009. The market launch was the culmination of more than 15 years of work that involved several collaborations. Some of the important early scientific work on peptide structure, for example, was carried out by a partnership with Harvard University and Massachusetts General Hospital. Expressed as a net present value, the cost of early drug discovery was the most expensive phase of development. When a drug is launched, companies try to recapture their investment in the price. But how do health systems realise value? Citing Denmark, Mr Andersen said the main players in the value chain are segmented. The healthcare system pays for patient treatments; employers sustain the cost of lost productivity and insurance companies pay for days lost due to illness. The system needs to be altered, he argued, by providing incentives for disease prevention. At the moment, no one is incentivised to pay for this. But they should be. This should be a key goal of personalised medicine.

Panel discussion

The panel discussion was led by Pierre Meulien, executive director of the Innovative Medicines Initiative.

Bianca Wittig, a medical director from AbbVie Inc, said the 'omics disciplines (eg genomics, proteomics and metabolomics) show how signalling pathways work, thereby providing a reservoir of new information for drug targets. What is missing are more companion diagnostics. These will need to be developed through collaboration and standardisation, and the use of registry data. Personalised medicine will require a new form of collaboration.

Raj Long, a senior advisor to the Bill and Melinda Gates Foundation, spoke about the challenges in neuroscience and the need for policymakers to create incentives for new drug development and reimbursement. Alzheimer's is an example of a disease where, despite much research, there still are no effective treatments. This is because the disease biology is not well understood. The research community needs to agree on what is known and not known and use existing tools to bridge the gap. Payers need to provide incentives for research and innovation, and regulators need to ask the question: if you put the patient in the middle, what are the essential risks and benefits? The emphasis should be on creating a single ecosystem to personalise the science around the patient.

Marisa Papaluca, senior scientific advisor at the European Medicines Agency (EMA), said there is a continuum from biomedical research to clinical practice to population health. The task is to join the dots among these phases in healthcare by harnessing new data sources and progressively changing the regulatory paradigm. EMA is already closing some of the gaps by engaging representatives from health technology assessment (HTA) bodies and patient groups in discussions about proposed medicines. But more could be done to help drug development, for example, by looking at models that simulate the performance of a drug in a preclinical setting to a clinical setting. Regulators could also be helped by more research on outcomes.

Brian O'Connor, chair of the European Connected Health Alliance (ECH Alliance) said that most experts in the field understand the problem; the issue is implementation. The ECH Alliance is a European digital health network. Mr O'Connor commended Scotland and Estonia for implementing digital health programmes. He told attendees: "Don't spend time worrying about the problems; implement what you can do at the moment."

Virginie Hivert, therapeutic development director at the European Organisation for Rare Diseases (EURODIS), said personalised medicine shares many of the features of orphan drug development. The key feature is bringing the patient into drug development at the right moment. The economic models guiding reimbursement also need to be revisited.

Catherine Larue, chief executive officer of the Luxembourg Institute of Health (LIH), said that innovation is not only a product, but can also be a new process. What is missing from the current initiatives in personalised medicine is a biomarker validation platform. The Integrated BioBank of Luxembourg, an autonomous institute within the LIH would be prepared to set up such a platform, she said. Founded in 2008, the biobank has more than 300,000 biological samples for use in applied medical research. The facility is certified by two separate quality management certification bodies.

Dr Meulien fielded questions from the audience which largely revolved around the question of how to influence the strategic decisions of pharma in relation to new drug development. In response, Peter Høngaard Andersen said that pharma needs incentives to develop drugs for small patient populations, where the return on investment is likely to be smaller than for the blockbuster drugs of the past. He suggested that companies receive a longer market exclusivity for personalised medicines. Marisa Papaluca pointed out that companies can elect to have parallel scientific advice at the European Medicines Agency with health technology assessment bodies. These discussions have helped companies get better value from their investments, which itself is an incentive to develop new drugs. Risk-sharing arrangements can also be a tool for helping companies realise an investment return, Raj Long said. This could mean the public authority commits to buying a medicine in advance, in exchange for a company's agreement to develop it. Dr Meulien concluded the discussions by saying that moving the personalised medicine initiative forward would be like "building a plane while it is flying."

CHALLENGE 5:
SHAPING SUSTAINABLE HEALTHCARE

Gaetano Guglielmi, Deputy Director General in the General Directorate for Research and Innovation in Healthcare in the Italian Ministry of Health, and Challenge Facilitator for IC PerMed Challenge 5 introduced the session. He said that personalised medicine represents a change in the paradigm of medicine similar to the introduction of antibiotics at the end of the second world war. Personalised medicine (eg, targeted therapies) have had a big impact on cancer, but the big question is cost. More collaboration among academia, industry and healthcare authorities will be required to address the issue of cost.

Continuing on the theme of cost, Walter Ricciardi, president of the National Institute of Health in Italy, said IC PerMed should focus on disease prevention. This may be the only way to reign in rising healthcare costs. Personalised medicine is a disruptive innovation. Inevitably this will mean adapting to a new set of values. Coincidentally, it corresponds with a change in demographics whereby older people are becoming a bigger proportion of the population, increasing healthcare costs. Disease prevention therefore should be a priority.

Frédérique Nowak, head of the biology, transfer and innovation department at the French National Cancer Institute, explained the challenge of implementing a personalised medicine strategy in an era of targeted cancer therapies. Since 2006, France has been providing molecular testing for all patients with cancer so that they can be prescribed with the most appropriate medicine as soon as possible. The cost of these tests is covered by the Institute and the Ministry of Health. Over the years there has been a progressive shift from one-on-one tests for individual patients to a next generation sequencing approach, which has increased the probability that an actionable mutation will be discovered in a patient's tumour. The cost of the programme has therefore increased because targeted therapies are now available for new subsets of patients. While this has posed challenges, France has nonetheless been able to integrate the stratification of patients into the healthcare system. But the system itself has to be flexible.

Andres Metspalu, director of the Estonian Genome Center at the University of Tartu, provided further information on the Estonian heathcare system which has been fully integrated with computers and registries. He said the system enjoys broad support from the population. The practical consequence is that if a citizen shows a risk for cardiovascular disease, he can do something about it by changing his lifestyle. Biobanks are an essential component of the system. There may also be a consequence for reimbursement. Just as consumers can return a pizza if it is poorly made, perhaps in future patients will be able to return a drug that does not work

Panel discussion

Mary Harney, a former minister for health and children in Ireland, led the panel discussion. The panellists included Maarten Ijzerman, Professor at the University of Twente in Enschede, the Netherlands; Varda Shalev, Director of the Institute for Health Research and Innovation, Maccabi Health Systems in Tel Aviv, Israel; Maria Aguirre Rueda, Director for Health Research and Innovation, Basque government, Spain; Roberto Salgado, Institut Jules Bordet in Brussels, Belgium and Matthias Perleth of the Gemeinsamer Bundesausschuss in Berlin, Germany.

In the discussion, panellists agreed that personalised medicine is an innovation that is here to stay. Technology is transforming the way that we understand the drivers of human health and disease. Using next generation sequencing, scientists can provide information on a person's genetic susceptibility to disease, or indeed help explain the drivers for good health. Together with information from the environment, it is possible to get a holistic picture of a person's health. The innovation goes further however and extends to healthcare systems. Using information technology, healthcare providers can collect, store and analyse data on whole populations making it possible to identify the most therapeutically effective, and cost effective interventions. Varda Shalev, director of the Institute for Health Research and Innovation in Tel Aviv, explained how Israel provides universal healthcare through a system of health maintenance organisations (HMOs) which compete on the basis of a basket of services delivered to patients. The new information technology makes it possible for data to be collected from patients, analysed and then used to design the best care. Similar IT-driven healthcare systems were described earlier in the conference by representatives from Scotland and Estonia.

Summarising the discussion, Walter Ricciardi, president of the National Institute of Health in Italy said there is a potential for Europe to be a leader in personalised medicine provided it makes rapid progress on systems for data collection and management, including progress in developing the electronic patient record. "We need to create a European ecosystem by building bridges between all parties," he said.

FORWARD-LOOK SESSION

The conference closed on 2 June 2016 with a discussion led by Peter O'Donnell, journalist, and including Ruxandra Draghia-Akli; Antoni Andreu of the Regional Ministry of Health of Catalonia, Barcelona, Spain; Maria Beatriz Da Silva Lima of the University of Lisbon, Portugal; Ilias Iakovidis of the European Commission, Ildikó Horváth of Semmelweis University in Budapest, Hungary as well as the IC PerMed Challenge Facilitators that had chaired the previous thematic sessions.

The theme of collaboration was picked up again in the final session. The term personalised medicine describes treatments for individual patients. But in fact the infrastructure that will be required to deliver this innovation is multinational and involves many disciplines. Professor Da Silva Lima, who is chair of the scientific committee of the Innovative Medicines Initiative (IMI), said the IMI can play its part by, for example, bringing experts together to develop databases to support personalised medicine. These in turn could be used to help industry design new clinical trials. Mr Iakovidis said it will be necessary to engage people across many sectors to advance personalised medicine. While we may know something about tailoring a medicine to a genetic profile, we know much less about how life styles and the environment affect health. "Can we do something practical on the nurture front," he asked. This might include helping older people become more literate about their health. The social media are important instruments for spreading information and cannot be ignored, the panellists said. These media are empowering patients and changing the relationships between patients and their doctors. They are also changing the way healthcare professionals do their jobs.

The following additional points were made:

- Europe needs a 'new ecosystem' for personalised medicine;
- The Eurobarameter survey might be used to find out how European citizens view their healthcare, and what they understand about personalised medicine.

- In designing the next steps, IC PerMed should study examples of best practice such as the integrated healthcare systems in Scotland and Estonia;
- New EU legislation on data protection needs to be examined to find out what its impact will be on personalised medicine;
- Elixir, an existing European infrastructure for life science information, should be used to advance personalised medicine;
- Biomarker research and validation efforts must accelerate so that they can be used in more clinical trials.

Dr Draghia-Akli summarised the action points by saying that in the short term IC PerMed should work on a new taxonomy of disease which would, in an iterative fashion, bridge the basic science to clinical research. Then, over the next five to seven years, it will be necessary to establish standards for electronic health records. The two initiatives could start in parallel, but the focus should be on achieving something concrete in the short-term.

CONCLUSION AND SUMMARY OF ACTIONABLE POINTS

The goal of personalised medicine is to put the patient at the centre of healthcare. How this will happen in practice is still open for discussion. The 1-2 June 2016 conference advanced these discussions for example by showing how integrated healthcare models are working in specific places – Scotland and Estonia – and how business models for personalised medicine can involve patients more directly in their healthcare.

IC PerMed will coordinate research, of the kind discussed at the Personalised Medicine Conference, and deliver on its vision to establish Europe as a global leader in personalised medicine research; support the personalised medicine science base; provide evidence of the benefits of personalised medicine and make this approach accessible to all citizens.

The following actionable points and comments, that can inform the continued work on IC PerMed, were identified by the conference rapporteur:

Challenge 1: Developing awareness and empowerment

- A changing demography is having an impact on the patient population. Policymakers need to find ways of specifically engaging older people in the management of their own health.
- The MIDATA cooperative model for data collection gives donors/patients real decision-making power. How would this model work across the EU?
- Patients should be consulted in the design of clinical studies, as well as the selection of research questions.
- HTA methodologies need to be reconfigured to take better account of the social value of medical interventions. What patients want from their medicines may be different from what the clinical trial outcomes tell the regulators.
- Apps should be developed to make it possible for patients to pose questions about their health and receive reliable answers;
- Regulatory discussions should incorporate the patient view. This already happens at the European Medicines Agency, but it might be implemented at the national drug agencies.
- More use should be made of patient-reported outcomes of treatments. This applies across the board from designing clinical trials to evaluating prospective new medicines to HTA.
- Patients should be engaged in the post-marketing surveillance of their medicines.

Challenge 2: Integrating Big Data and ICT solutions

- There is a need to make progress in developing and implementing the electronic patient record. In many countries this is happening at a hospital level. In Scotland and Estonia the e-record is integrated into the healthcare system. What should the model for the EU be? Should all citizens of the EU also have a unique ID and a patient record, or does that go too far? More clarity on the form and purposes of the electronic patient record is required.
- The Farr institute in the UK links electronic health data with other forms of research to build capacity in health informatics. Something similar could be created at EU level.
- Computer capacity needs to be increased across the EU. The EU will create a European science cloud which can address this issue.
- Issues relating to the ownership and management of data need to be clarified, possibly in legislation. Steps also need to be taken to ensure data quality and maturity and standardisation. Rules should also ensure that data can be shared across borders.
- There is a need to revisit the issue of consent. Are current methods for obtaining consent from patients adequate for the new digital age? If a large company acquires a smaller company giving it access to patient records for the first time, can it assume that the patient consent is transferable? What if the new owner has a different plan for the use of this data than the previous owner? Are current methods for obtaining consent from children and the elderly adequate? If a patient has consented to the use of his/her records in a biobank, can that consent be withdrawn at any time?
- The model of the data cooperative should be investigated further for its possible application across Europe.

- Consideration should be given to completing the infrastructure started by Elixir with an Institute for Biomedical Data and Informatics in each member state. The EU could then launch a project to join up these institutes.
- Strategies are needed to make sense of existing and newly collected data.
- Examine how existing infrastructures under the European Strategy Forum on Research Infrastructures (ESFRI) can be used to meet the needs of personalised medicine research.

Challenge 3: Translating basic to clinical research and beyond

- The healthcare systems of the EU need to shift towards putting a bigger emphasis on disease prevention. Scotland and Estonia show how this can be done. Could this be applied across the EU as a whole?
- Patient-reported outcome statistics are essential to the new paradigm. They need to be collected and analysed for use in trial design, regulation and reimbursement.
- The value of genomic data is well understood. But for translation, there also needs to be a better understanding and use of phenotypic data. The phenotypic variations among individuals need to be explained and how this affects their responses to treatment.
- More attention should be paid to the potential for improving existing treatments and finding new ones through the retrospective analysis of clinical trials. Biobanks should be a standard feature of clinical trials, to enable these analyses to take place.

Challenge 4: Bringing innovation to the market

- The clinical descriptions of diseases need to be standardised to enable a better comparison and analysis of data.
- Biobanks need to be fit for purpose; not all of them are.
- There is a need for an EU platform for biomarker development and validation, possibly building on the EMA biomarker qualification programme.
- A European research infrastructure is important, but there also needs to be a local infrastructure for defined projects.
- Business models need to be explored that reward research strategies for disease prevention.
- There is a need for more diagnostic tools, perhaps developed in research collaborations.
- A risk-sharing model for the development of new personalised treatments in difficult diseases should be explored. This could include the development of biomarkers to differentiate responders from non-responders.
- Computer models that simulate early clinical trials should be explored. This could be an efficient way of predicting the success, or failure, of a drug in the clinic.
- The EU could consider launching a programme akin to national programmes such as the UK's 100,000 Genomes Project which links genomic and clinical data in order to develop new drugs for cancer and rare diseases.
- The role of wearable sensors in disease management needs to be clarified.
- Regulators could be helped by more patient-related outcomes research.

Challenge 5: Shaping sustainable healthcare

- The French molecular testing programme for cancer patients should be reviewed for possible wider application.
- Economic models for HTA need to be revisited, considering the wider patient perspective.
- Electronic patient records, registries and biobanks will all contribute to sustainable healthcare in the future.
- Data quality and standardisation are critical for going forward.

This report is a summary of the presentations and discussions which took place at the Personalised Medicine Conference 2016 on 1-2 June in Brussels. Personalised medicine is an approach to healthcare that puts the citizen in the centre. By developing tailor-made diagnostic, treatment and prevention strategies, patients receive therapies that specifically work for them. It also allows people to participate in the management of their own health by having access to information about the prevention and treatment of disease. The Personalised Medicine Conference 2016 aimed to discuss personalised medicine through a research policy lens. It moreover presented a new initiative, bringing together health research funding and policy making organisations, called the International Consortium for Personalised Medicine (IC PerMed).

Studies and reports

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