ICPerMed Conference – Prelude to the Future of Medicine

The International Consortium for Personalised Medicine – ICPerMed, organised, with the support of the French National Research Agency (ANR), a hybrid conference entitled "ICPerMed Conference – Prelude to the Future of Medicine" in Paris on October 5-6, 2022.

Personalised medicine aims to deliver the right treatment to the right patient at the right time by relying on specific characteristics of individuals such as information on the lifestyle of patients and citizens or omics data (information or molecular characteristics of a cell or population of cells) in order to better understand the cellular mechanisms that may be disrupted in a given pathology. This includes genomic data for the study of the genome, transcriptomic data for the study of gene expression, proteomic data for the study of proteins etc.

If modern medicine already takes into account the specificities of patients, personalised medicine promises to push this principle much further. It aims to offer tailor-made diagnoses and treatments for everyone, thanks to a detailed knowledge of their health journey, but also of their lifestyle or even their genome. With the increasing knowledge on biological mechanisms underlying diseases and the emergence of Big Data and new technologies, personalised medicine allows to improve diagnostic and risk prediction tools, to develop more targeted therapeutic strategies, to enhance therapeutic decision-making and to implement effective prevention measures.

To become a reality, personalised medicine needs to democratise and to transfer the results obtained in the laboratory into practice. To this end, ICPerMed aims to decipher obstacles and challenges faced for the implementation of personalised medicine and promote solutions facilitating it. That is the reason why ICPerMed tries to establish a common understanding concerning the cost-benefit value for the health system, to engage patients and citizen in their own health, to develop the use of health data and promote innovative ecosystems connecting research, technology and innovation and ensuring scientific breakthroughs can make it to the market. This implies to call on many fields such as data science, human and social sciences, health economics, public health or health regulations and to promote multi- and transdisciplinary approaches.

The ICPerMed conference was structured around 4 keynote lectures, offering a focus on promising examples of targeted treatments or a personalised approach for diagnosis, and around 5 sessions addressing crucial aspects for the successful implementation of personalised medicine.

The promises of single-cell technologies in personalised medicine

Prof. Nikolaus Rajewsky from MDC Berlin presented how rapidly developing single-cell technologies together with Artificial Intelligence (AI) and patient-derived disease models are revolutionising our understanding of diseases and therapy response in patients at the cellular level and offer new opportunities for personalised medicine. Cell-based interceptive medicine leverages these technologies to develop new solutions for the early detection of disease and identifies new therapy targets that will enable diseases to be intercepted before they progress.

Genome editing approaches and gene therapies are becoming a reality for the treatment of genetic diseases

Dr. Annarita Miccio from the Imagine Institute of genetic disease in Paris and Francesca Ferrua from the San Raffaele Hospital in Milan presented us examples of gene therapies for β-hemoglobinopathies as Sickle cell disease (SCD) or β-thalassemias and for adenosine deaminase deficiency causing severe combined immunodeficiency (ADA-SCID), respectively. Gene therapies as Strimvelis for ADA-SCID aim to provide the organism with the correct copy of a faulty gene or another gene that can compensate or its malfunction using viral vectors. Recently, genome editing strategies have been developed to correct the genetic defect or target disease modifiers in patient cells and provide an effective treatment. Currents studies will likely further improve the safety of these strategies, simplify the gene therapy procedure and reduce the costs, thus allowing the wide use of these therapies.

Antisense oligonucleotides, the next frontier for personalised medicine treatments

Dr. Erik de Vrieze from Radboud University Medical Center in The Netherlands presented an example of a recent RNA technology called antisense oligonucleotides for otogenetic disorders in Usher syndrome and DFNA9 hearing loss. Antisense oligonucleotides are short, synthetic single-stranded RNA that can alter RNA and reduce, restore, or modify protein expression through several distinct mechanisms. Dr. de Vrieze highlighted that manipulating gene expression with antisense oligonucleotides provides countless possibilities to develop precision medicine for disorders with a defined genetic target. His work on antisense oligonucleotide-based treatments for Usher syndrome and DFNA9 are perfect examples of this.

The adaptation of clinical trials to meet the specificities of personalised medicine

Personalised medicine is a key driver for innovative clinical trials design and clinical development and allows more flexible and efficient trials. With the presentation of the I-SPY 2 adaptive clinical trial for breast cancer, it has been shown how the design allows to accelerate the development, the access to treatment and facilitate regulatory procedures. However, for confirmatory adaptive design trials, clinicians need to be clear on the questions they want to answer and be careful with the complexity of the design to deal with decision rules and power calculation. Additionally, adaptive clinical trials are not the only tool in personalised medicine toolbox and can now be complemented with observational data. Both observational studies and randomized controlled trials (RCTs) are mutually complementary in evaluating effectiveness of treatment. And while observational studies have a lower internal validity compared to RCTs, they can evaluate effectiveness in real-world conditions and provide information on subgroups of patients where RCT evidence is not available.

Personalised medicine and health economics – Can they be reconciled?

Recent evidence on cost-effectiveness shows personalised medicine has great potential to better allocate scarce resource by redesigning patient pathways. In this session, speakers explained how the results of cost-effectiveness analysis is necessary but not sufficient evidence to move precision medicine into clinical practice for the benefit of patient populations. Other methods used by health economists, such as studies to identify and measure the preferences of patients, clinicians and the public and accounting for heterogeneity

in possible cost-effectiveness in sub-groups of populations, are also key pieces of evidence needed to enable the implementation of precision medicine into healthcare systems.

The challenges of health data governance

The session focused on health information and data and brought together a local example at the hospital level as well as national and international cross-border perspectives.

The session was the occasion to discover the Health Data Hub, a unique gateway to health data in France providing a collection of databases including medico-administrative data, offering secure technological platform and tools bringing together key stakeholders.

While the presentation of TEHDAS outlined the strategy of the European Health Data Space aiming at offering data access within the EU and across sectors, ensuring European rules and values are fully respected. TEHDAS aims to support the Members States and the European Commission to develop and promote concepts for sharing of data in secondary use: governance, data quality, infrastructure, citizen engagement, data altruism and sustainability.

Finally, Prof. Sang-Heon Lee, head of the Medical Science and Information Unit at Korea University Medicine, presented a cloud-based hospital information system (HIS) that has been operating in three Korea University hospitals. He pursues active collaborations with researchers and AI companies to build better AI models using big medical data accumulated via the cloud-based HIS.

Understanding patient's perspective in personalised healthcare

Personalised medicine is radically changing care pathways, patient-physician relationship and how patients perceive their disease. In personalised healthcare systems, the perspective of patients on how they experience and understand the results of sequencing-based diagnostics is then essential to apprehend to improve care and satisfaction. Patients and their families hold pieces of the jigsaw that can help to find new diagnoses. As parents they are among the most motived people in the world to learn new stuff it affects their children and to drive progress forward. It is crucial to create realistic expectations to what genomic medicine can achieve and offer and what it cannot. As for a healthcare organisation, it is essential to involve patients and family members from an early stage, to pay for their time, to consider their recommendations and adjust your plans accordingly toward a long term relationships.

The creation of ecosystems supporting innovation in personalised medicine

Creating adequate environment facilitating public-private partnerships and encouraging intersectoral and multidisciplinary collaborations is crucial to accelerate the transfer of research results to the market. The Sheba Medical Center, Israel's largest hospital, and its related innovation programme ARC (Accelerate, Redesign and Collaborate) is an excellent example of this at the local scale on how to redesign healthcare, transform healthcare delivery and improve patient care while being more effective, efficient and equitable. To this end, they nurture international collaboration uniting leading Academic Medical Centers with innovative start-ups and strategic industry partners to promote accelerated development and implementation through the sharing of data and streamlined collaborations. While Norway is building a

comprehensive national initiative with public funding for increasing access to precision treatment by connecting all stakeholders from research, clinics, data, regulatory authority and by stimulating public-private collaborations to address key obstacles and pilot novel solutions. Finally, the Vanguard initiative example demonstrates how important the role of European regions is and how the concept of smart specialisation could leverage regional assets and resources for the need of implementation of personalised medicine.

European infrastructures acting as strong assets for the development and implementation of personalised medicine

New for this event edition, ICPerMed gave the floor to members of its Stakeholder Forum that allow diverse stakeholders in personalised medicine as research organisation, industry, health care provider, payer/insurance, patient organisation etc. to participate into ICPerMed activities and share ideas and best practice examples or identify research priorities and bottlenecks. During this Stakeholder Forum Session, ICPerMed was pleased to welcome 3 European infrastructures: ECRIN, EATRIS and EU-AMRI that are decentralised with national hubs and nodes of participating Member States. They provide services and access to infrastructures to the research community as for instance developing joint standards, counselling and training on quality management, Ethical, Legal and Social Aspects (ELSA), data protection, and provide access to infrastructure instruments to samples and data.

ECRIN – The European Clinical Research Infrastructure Network – as a research infrastructure fosters the development and implementation of personalised medicine research by supporting personalised medicine clinical trials, leading methodological research in personalised medicine to ensure excellence, robustness, reproducibility and acceptability of personalised medicine studies and helping to strengthen international personalised medicine research networks.

EATRIS – The European infrastructure for translational medicine – aims to accelerate the translation of research discoveries into patient benefit by notably providing access to research services and expertise to industry, academia and researchers funders and by developing new research tools as the Multi-Omic Toolbox. The Multi-Omic Toolbox is an open access resource containing Standard Operating Procedures, guidelines for best practices, reference materials, quality parameters, data analytical tools, repository of multi-omic data.

EU-AMRI – European Alliance of Medical Research Infrastructure – is a collaboration between EATRIS, ECRIN, and BBMRI (Biobanking and BioMolecular resources Research Infrastructure). The three research infrastructures work in parallel to provide complementary services to researchers in the field of biomedical sciences and support the development of personalised medicine and new treatments. EU-AMRI is not just a collaboration; it is establishing a framework for long-term cooperation building a constellation of services for accelerating patient-centric biomedical research.

You can find the presentations and video recordings here.

Photo gallery

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About ICPerMed

The International Consortium for Personalised Medicine (ICPerMed) brings together European and international partners from 50 funding organisations and 29 ministries representing countries and the European Commission (EC). ICPerMed works on coordinating and supporting research to develop personalised medicine approaches. Thereby, the central aim of ICPerMed is to align and encourage joint efforts in personalised medicine research and implementation on a European and international level.

The European Commission and Personalised Medicine

The European Commission has been supporting research in personalised medicine since its Seventh Framework Program (FP7, 2007-2013). Its Horizon 2020 program was also accompanied by an envelope of more than three billion euros for such projects. The European Commission thus wishes to strengthen research and innovation activities, while reducing the duplication and fragmentation of investments, in line with the United Nations' sustainable development objectives and by informing decision-makers, in order to enlighten them in the development and implementation of new policies and regulations.