Personalised Medicine: an EU Perspective

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Personalised medicine: towards a definition

"Personalised medicine refers to a medical model using molecular profiling 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"
Healthcare opportunities: making medical treatments more personalised

- Avalanche of new –omics and molecular information following the sequencing of the human genome
- Translation of –omics from basic to clinical research can bring us better understanding of health and disease

Need innovative approaches for changing the focus from treatment and cure to prediction and prevention
Personalised medicine to address significant challenges...

- Loss of €35 trillion over next 20 years worldwide due to non-communicable diseases
- Increasing pressure on European healthcare systems
- Current medical treatments in many cases not effective
- The EU is not closing gap with global innovation leaders
- Biomedical companies are finding drug development in Europe challenging
...and benefit from opportunities

- Stratified and personalised medicine can deliver better outcomes for patients and potential cost savings
- Studies suggest cost savings of 37% for breast cancer and 46% for CVD when a stratified approach is taken
- Europe can lead implementation of personalised medicine thanks to favourable conditions
Personalised Medicine: preparing the ground

- 2010: Preparatory workshops (-omics, biomarkers, clinical trials/regulatory, uptake)
- 2011: European Perspectives conference
- 2013: "Omics report"
- Identify key challenges to be addressed by research
Staff Working Document on
Use of '-omics' technologies in the development
of personalised medicine

• the potential for, and issues with, the use of
'-omics' technologies in personalised medicine,
and the related EU research funding

• recent developments in EU legislation for placing
medicinal products and devices on the market

• factors affecting the uptake of personalised
medicine in health care systems

Available on:
The framework for Personalised Medicine

R&D the basics

R&D stratifying tools

R&D test in human

Towards the market

Uptake in healthcare

In patients

“Omics” Technologies Data Samples Statistics

Biomarkers Identification Qualification Validation Data modelling tools Technical aspects & challenges

Clinical trials Methodologies Ethics Patient - recruitment

Diagnostics & Therapies Approval processes Regulatory aspects

Pricing & Reimbursement Health economy HTA Novel models of healthcare organisation

Availability & usability in the clinic Patient perspective Equal treatment Training of professionals

Prediction - Prevention – Treatment - Cure
Identified key research challenges

Breaking barriers & speaking the same language
"cross-disciplinarity", capacity building, education & training

Generating knowledge & developing the right tools
standards, clinical bioinformatics, adaptation of tools

Translating knowledge to medical applications
disease taxonomy, biomarker validation, clinical trials

Understanding the value & economic aspects
health care pilots, HTA, comparative effectiveness research, value chain
EU Health Research Programme: Enabling personalised medicine 2007-2013

- Large scale data gathering and "-omics"
- Technology development
- Diagnostics
- Biomarkers
- Pre-clinical and clinical research
- Rare diseases: small patient populations
- Public health research
- IMI projects with pharma industry
## Example: Overview 2007-2013

**Large-scale data gathering**

<table>
<thead>
<tr>
<th>Resources</th>
<th>Basic knowledge</th>
<th>International cooperation</th>
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<tbody>
<tr>
<td><strong>Genetic epidemiology</strong></td>
<td>genetic/genomic epidemiology</td>
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<td></td>
<td>genetic epidemiology of disease</td>
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<td></td>
<td>(hypertension, infections, pre-eclampsia...)</td>
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<tr>
<td><strong>Genomics &amp; other -omics</strong></td>
<td>epigenomics</td>
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<td>cancer genomics</td>
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<td>metagenomics</td>
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<td>proteomics standardisation</td>
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<td>proteomics</td>
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<td>lipidomics</td>
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<td><strong>Structural biology</strong></td>
<td>receptors, channels &amp; transporters, signalling proteins</td>
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<td>and Cell biology</td>
<td>Stem cells</td>
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<td><strong>Model organisms</strong></td>
<td>zebrafish, fly, mouse (IKMC), rat &amp; dog as models for human disease</td>
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35 projects
€ 351 million
Example: Overview 2007-2013
Large-scale data gathering

Contributing to international cooperation

- International Human Microbiome Consortium
- International K.O. Mouse Consortium
- International Rare Diseases Research Consortium
- Global Alliance for Chronic Diseases
- International Cancer Genomics Consortium
- International Initiative for Traumatic Brain Injury Research
- Global Research Collaboration for Infectious Disease Preparedness
- International Human Epigenome Consortium
Main achievements

- Structuring research areas within EU and beyond
- Setting standards of high quality data collection, integration and analysis
- Open access data repositories

Project example: BLUEPRINT

- BLUEPRINT preliminary results enable discovery of causative epigenetic factor for rare blood disease (TAR syndrome)
Example: clinical trials for rare diseases

- 3 projects bringing together international experts in innovative clinical trial design methodology along with key stakeholders
- Innovative statistical design methodologies for clinical trials in small populations focusing on rare diseases
Example: "-Oomics" for rare diseases

- 2 projects focusing on molecular characterisation of a large group of rare diseases using -omics technologies.
- Ontologies, reference -omics profiles, diseases models, development of technologies
- New means to diagnose and allow development of new treatments for these diseases
- Platform for integrating -omics data with clinical data, connecting registries, biobanks and clinical bioinformatics
- Supporting collection and storage of data and samples in EURenOmics and NEUROMICS
- Provides access to -omics profiles and samples
Supporting policy development
• The EU’s 2014-20 programme for research & innovation (around € 80 billion)

• A core part of Europe 2020, Innovation Union & European Research Area

• Three priorities: Excellent science, Industrial leadership, Societal challenges
Health, demographic change and wellbeing challenge

- Translate science to benefit citizens
- Test and demonstrate new healthcare models, approaches and tools
- Promote healthy and active ageing
- Improve health outcomes, reduce inequalities
- Support a competitive health sector

Over €7 billion to health research
Focus areas of 2014-2015 Work Programme

- Understanding health, ageing & disease
- Improving diagnosis
- Innovative treatments and technologies
- Improving health information, data exploitation and providing an evidence base for health policies and regulation
- Effective health promotion, disease prevention, preparedness and screening
- Advancing active and healthy ageing
- Integrated, sustainable, citizen-centered care

Implementing personalised medicine in healthcare settings
PHC 5 – 2014: Health promotion and disease prevention: translating ‘omics’ into stratified approaches

- develop and assess a personalized / stratified health promotion or disease prevention programme, taking into account the ‘omics’ characteristics of individuals, complemented by environmental and/or lifestyle factors
PHC 24 – 2015: Piloting personalised medicine in health and care systems

- Pilots of new models of care, based on the concept of personalised medicine
- Proposals should ensure coordination with national, regional or local authorities engaging in health sector reform
- Evidence for a validated model of organisation of care based on the concept of personalised medicine should be produced
A Strategic Research Agenda for Europe

- Inclusive focus on key challenges across development/value chain
- Importance of multidisciplinary research
- Importance of implementation aspects
- Pilots and proof of principle
  - Evidence of clinical utility
  - Evidence of value
Thank you

www.ec.europa.eu/research/health
www.ec.europa.eu/research/horizon2020