European challenges and the way forward”

EMA Human Scientific Committees’: (PCWP) and (HCPWP)

Joint meeting
September 20th, 2016

Denis Horgan, EAPM Executive Director
## Technology Advancement

<table>
<thead>
<tr>
<th>Year</th>
<th>2005</th>
<th>2015</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost of Sequencing a Human Genome</td>
<td>$100 million</td>
<td>~$1000</td>
</tr>
<tr>
<td>Personalized Medicine Products on the Market</td>
<td>14</td>
<td>&gt;160</td>
</tr>
<tr>
<td>Personalized Medicine U.S. Drug approvals that year</td>
<td>1</td>
<td>13</td>
</tr>
<tr>
<td>Example of Time Elapsed from Discovery to Market</td>
<td>26 years (EGFR → cetuximab)</td>
<td>4 years (ALK → crizotinib)</td>
</tr>
</tbody>
</table>

- **42% of All Drugs in Development Are Personalized Medicines**
- **73% of Oncology Drugs in Development Are Personalized Medicines**
The Perfect Storm?

Regulatory Initiatives
Changing Science
Increasing Data Volume, Variety & Velocity
Shifting Reimbursement Landscape

Traditional Approaches are Limited

Point Applications
- Limited to single use case
- Not highly scalable
- Reinforces silos

Homegrown Tools
- Challenge keeping pace with industry best practices
- High operational and opportunity costs

Established Solutions
- Often not open platforms
- Incomplete Electronic Data Warehouse models
- Inflexible approaches
Healthcare System

Bringing healthcare to the next level requires that we move out of our castles and work together.
Personalised Medicine

• Is it not just a natural evolution?
• We understand more about disease; so shouldn’t our treatment approaches be more complex?
• Where are we?
• What are the barriers?
Personalised Medicine

- Science has evolved but health systems lag behind

- We understand more about disease; so shouldn’t our treatment approaches be more complex?

- Challenges for society
We Are Getting Faster!!!
What?
• pan-EU network on personalised medicine and personalised healthcare
  (1) capacity-building (connecting existing initiatives)
  (2) coordination (contributing to policy roadmap based on needs)
  (3) open platform (sharing best practices and giving policy advice)
  (4) networking opportunities

Why IAPM?
• fragmented picture in European and Polish landscape: need for overarching initiative

Why now?
• expressed need for timely guidance for implementation on European level
  (good governance = branding/trust developed by with all stakeholders)
Different Legislative Issues Addressed by EAPM

- Informed Consent
- Research in Personalised Medicine
- Establishing consistent language
- Ensuring access for tests AND treatment
- Bio-banking
- Patient empowerment
- Value/Cost Rubicon
- Expanding organizational reach to untapped patients
- Genetic, genomic and NGS testing
- Expanding access for tests AND treatment
- Collaborating with your healthcare team
- Treatment decision-making considerations
- Registeries
- Training policymakers
- Clinical Evidence
- Defining
- Role of Ethics Committee

EAPM: European Alliance for Personalised Medicine
Chair: INTEL

Policy Ask: “By 2020, the EU should endeavour to achieve widespread benefits for patients and citizens from personalised healthcare by defining in 2015, and subsequently executing a Data Strategy for Personalised Medicine.”

Chair: Queen’s University Belfast

**Key Policy Ask:** For the EU to commit to the development of a European Translational Research Platform that enables the efficient translation of research discoveries to innovative diagnostics, therapeutics, products and processes that will benefit European patients, industries and societies.

Chair: European Haematology Association

**Key Policy Ask:** “By 2020, the EU should support the development of a Europe-wide education and training of healthcare professionals’ curriculum for the personalised medicine era, by committing to this in 2015. The EU should subsequently facilitate the development of an Education and Training Strategy for HCP in Personalised Medicine.”

Chair: ROCHE & European Patient Forum

**Key Policy Asks:** to ensure:

a) health care resources allocated to development and utilisation of personalised medicine, through acceptance of its long-term cost-effective benefits and

b) to effect a paradigm shift in pricing and reimbursement to recognise the societal value of a medicine.
Three dimensions

European healthcare challenges

- Increase in the incidence of chronic disease
- Change in demographics and epidemiology
- Non Compliance
- Patient and citizen needs and involvement in the healthcare ecosystem

Research and Development (technological/Scientific) challenges

- How does this impact R&D?
- What solutions can be drive through R&D?

Regulatory/healthcare delivery challenges

- How does this impact healthcare delivery?
- What research is required to provide solutions?
No commonly agreed definition of the term “personalised medicine”.

**Widely understood that personalised medicine refers to a:**

• **medical model using characterisation** of individuals' phenotypes and genotypes (e.g. molecular profiling, medical imaging, lifestyle data) for tailoring the right therapeutic strategy for the right person at the right time, and/or to determine the predisposition to disease and/or to deliver timely and targeted prevention.

• Personalised medicine relates to the broader concept of patient-centred care, which takes into account that, in general, healthcare systems need to better respond to patient needs;
Growing divide across Europe
“An imbalance between rich and poor is the oldest and most fatal ailment of all republics”

Disparities in male life expectancy between East and West Europe have grown in the last 40 years

Public health efforts are at risk due to poor European and National legislation

Health services for children are not keeping pace

Migration of health workers is a major threat to many European countries

Economic policy has major effects on health

Economic disparities are a balance between what we spend on cancer care and what cancer ‘costs’ economies

Massive variation in direct spend on cancer care across Europe: *major differences in where money is spent*

Healthcare costs per incident COLORECTAL cancer, adjusted for price differentials

- Lithuania: €8,421
- Portugal: €9,002
- Hungary: €9,177
- Sweden: €9,325
- Bulgaria: €9,600
- Denmark: €9,891
- Cyprus: €10,081
- Romania: €11,140
- Latvia: €11,314
- Belgium: €11,410
- Slovenia: €12,215
- Italy: €12,526
- Malta: €13,642
- France: €14,459
- Spain: €14,623
- United Kingdom: €15,955
- EU-27: €16,243
- Slovakia: €16,478
- Czech Republic: €17,175
- Ireland: €18,072
- Estonia: €21,036
- Netherlands: €21,301
- Poland: €21,475
- Germany: €22,887
- Austria: €23,476
- Luxembourg: €24,381
- Finland: €24,646
- Greece: €26,215

European Alliance for Personalised Medicine
Medicines have contributed to 15% of increased health costs in Europe – with hospitalizations and elderly care being the key drivers.

Share of Growth per healthcare category (2004 – 2010, 15 EU OECD Countries, population-weighted, current prices, PPP, $)

Note: Countries included: Austria, Belgium, Czech Republic, Denmark, Estonia, Finland, France, Germany, Hungary, Netherlands, Poland, Portugal, Slovakia, Slovenia, Spain, Sweden

Source: OECD Health Statistics Database (accessed 2013); Eurostat Database (accessed 2013)
Total cost 2 billion euro

The bridge over Öresund between Sweden and Denmark
Personalised and Precision Politics

• President Obama’s **Precision Medicine Initiative**
• European Commissions Work Programme for **Health, Demographic Change and Wellbeing**
• Genomics England **100,000 Genomes Project**
• MRC **Stratified Medicine Initiative**
• Vice President Biden’s **Cancer Moonshot**
• **Innovative Medicine Initiative**
Council Conclusion on PM

- Big Data
- Research
- Patient Access
- Health Literacy
- Education & training
- Regulatory Issues

OUTCOME OF PROCEEDINGS
From: General Secretariat of the Council
On: 7 December 2015
To: Delegations
No. prev. doc.: 14393/15
Subject: Personalised medicine for patients
   - Council conclusions (7 December 2015)

Delegations will find in the annex the Council conclusions on personalised medicine for patients, adopted by the Council at its 3434th meeting held on 7 December 2015.
What we got: 2016 Council Conclusions on PM

- HCP education
- Public education
- Specialists collaboration
- Best practices & MS dialogue
- HTA, STAMP
- GDPR
- Promote H2020 results
- Study on Big Data for PM
- Leverage ERNs for R&D
- Access to PM therapies
- Use genomics for public health

Data standards, collection, sharing and processing inc biobanks and EHRs
BUT, healthcare systems …

- Function nationally

- Have national efficiency as their highest priority
Developing diagnostics for Personalised Medicine

What is the ‘right’ level of clinical evidence for a companion diagnostic?
- Balance of scientific rigour and access to innovation

Should lab developed tests have to meet the same criteria as other companion diagnostics?
- If not, what Quality Assurance and audit measures should be in place?
- Who should oversee this process?

How do you incentivise innovation in diagnostics?
- Diagnostic platform technology moves quickly (eg next generation sequencing)
- Innovator test may be quickly superseded
- Data exclusivity is problematic
- Clinical data / tissue samples availability to provide clinical evidence

How should diagnostics be reimbursed?
- Fee for service?
- Value of the information to patient / doctor / health care system?

The future of companion diagnostics
- Panels of markers, not individual tests
Navigating the legislative Barriers
Patient John Doe has Diabetes Type II (ICD10 = E11.1)

I don’t use ICD??? MeSH code for diabetes is C18.452.394.750

Did he say John and Jane Doe?
The political context

The whistleblower

I can’t allow the US government to destroy privacy and basic liberties
The European Parliament’s position

- LIBE committee voted in October 2013
- 91 compromise amendments from over 3000 tabled
- Block vote of 85 amendments
- Almost unanimous in favour of the amendments because agreed by political groups in advance.
Impact

- Three major pieces of legislatives revised
- Clinical Trials Regulation
- Data Protection Regulation
- In-Vitro Diagnostics
Setting the Personalised Medicine (PM) Agenda

• Significant successes that have been practice changing¹
• Provide real hope for PM integration
• BUT
• Fragmentation, Silo Mentality and other Barriers threaten its translation into National Health Systems ², ³

³ Use of ‘omics technologies in the development of personalised medicine EC 2013
Key Stakeholders

Personalized Medicine

- Researchers ✓
- Dx Industry ✓
- Rx Industry ✓
- Providers
- Patients
- Payers
- Regulators ✓

Diagnostics & Treatment
What are companion diagnostics?

Companion diagnostics (CDx) = specific group of in vitro diagnostic tests providing information that helps determine a patient’s response to a targeted therapy

Benefits of CDx:

- certainty on the potential benefit of a treatment/
- reduces inefficient use of healthcare resources while optimizing patient outcomes

EXPENDITURE:

Expenditure on CDx: accounts for far less than 1% of the total healthcare expenditure
How interested would you be in having a test done that would provide you/your doctor with information to guide therapy choices for cancers, infectious diseases, neurological disorders (e.g. Alzheimer’s disease), and other conditions, while also avoiding unnecessary treatments with little chance of success and side effects?

<table>
<thead>
<tr>
<th>Interest Level</th>
<th>Percentage</th>
</tr>
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<tbody>
<tr>
<td>Very interested</td>
<td>85.71%</td>
</tr>
<tr>
<td>Slightly interested</td>
<td>10.71%</td>
</tr>
<tr>
<td>Neither interested nor disinterested</td>
<td>0.00%</td>
</tr>
<tr>
<td>Not very interested</td>
<td>3.57%</td>
</tr>
<tr>
<td>Definitely not interested</td>
<td>0.00%</td>
</tr>
</tbody>
</table>
What message would you send to policymakers and politicians regarding your interest and needs for knowing your health stats, having a diagnosis in General, and access companion diagnostic information treatment?

Key quotes

- “Research is not the bottleneck, reimbursement is.”
- “Avoidance, including the diagnostic, should become the first goal to reach in ensuring accessibility.”
- “Being able to predict illness would surely be financially and medically sound.”
Healthcare System

Bringing healthcare to the next level requires that we move out of our castles and work together.
Geographical scope

Italy
Poland
Spain
France
Germany
All-Ireland
United Kingdom
Bulgaria
Romania
Sweden
FUTURE OF PERSONALIZED MEDICINE

Better evidence for diagnostics and therapies

Translate research

Empower patients!

Genetic counsel + decision support + genetic literacy

Need more agile regulatory system

Giant leaps in medicine are just around the corner!

Test before you treat

Give me my data!

Get to the right drug the first time!

Take care of your own health!

All of the data from the internet can be stored in DNA in a small test tube.
Thank you!

European Alliance for Personalised Medicine

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