



European Alliance for
Personalised Medicine



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

2005		2015
\$100 million	Cost of Sequencing a Human Genome	~\$1000
14	Personalized Medicine Products on the Market	>160
1	Personalized Medicine U.S. Drug approvals that year	13
26 years (EGFR → cetuximab)	Example of Time Elapsed from Discovery to Market	4 years (ALK → crizotinib)

42% of All Drugs in Development Are Personalized Medicines



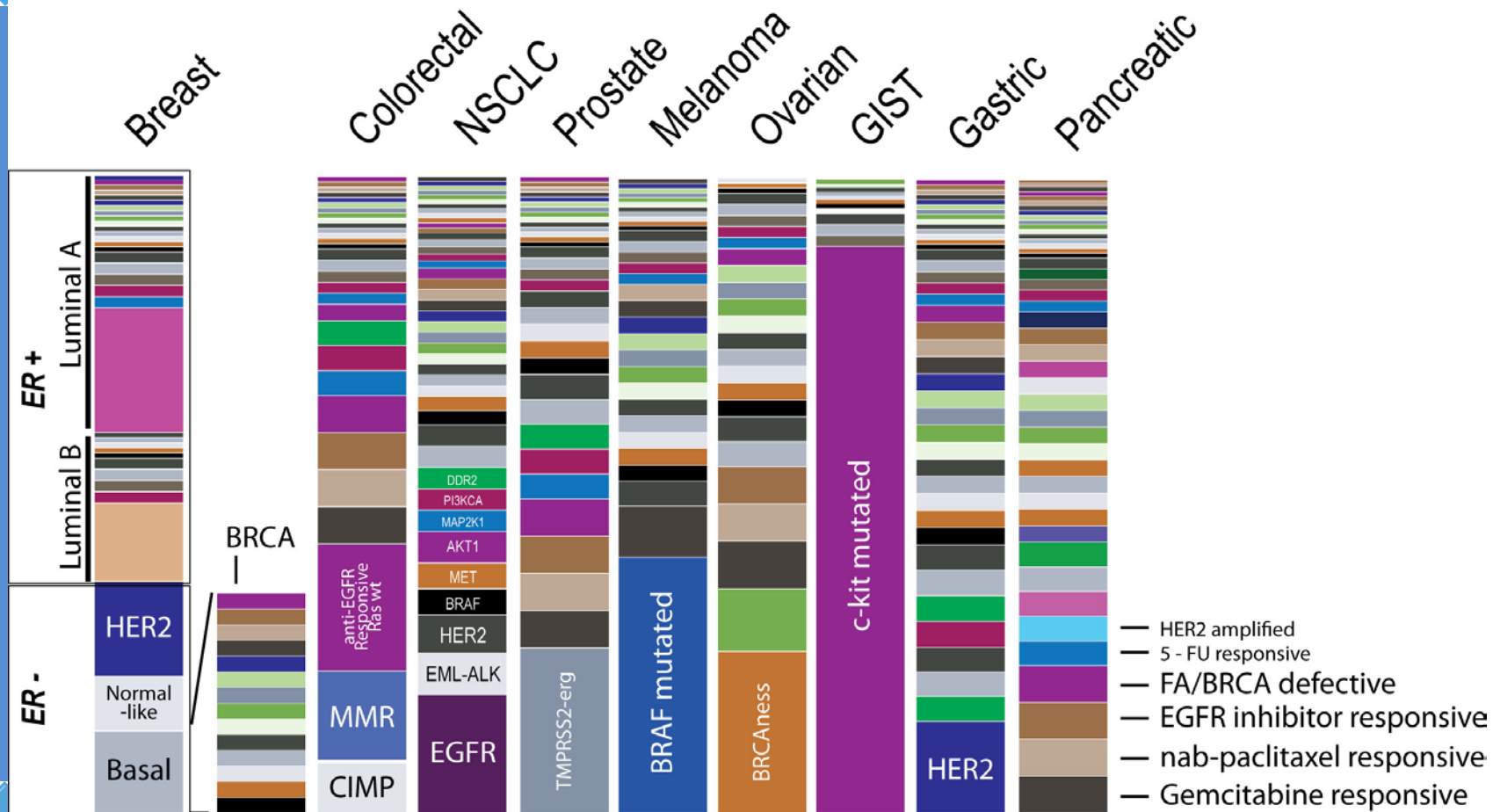
Personalized Medicines

73% of Oncology Drugs in Development Are Personalized Medicines



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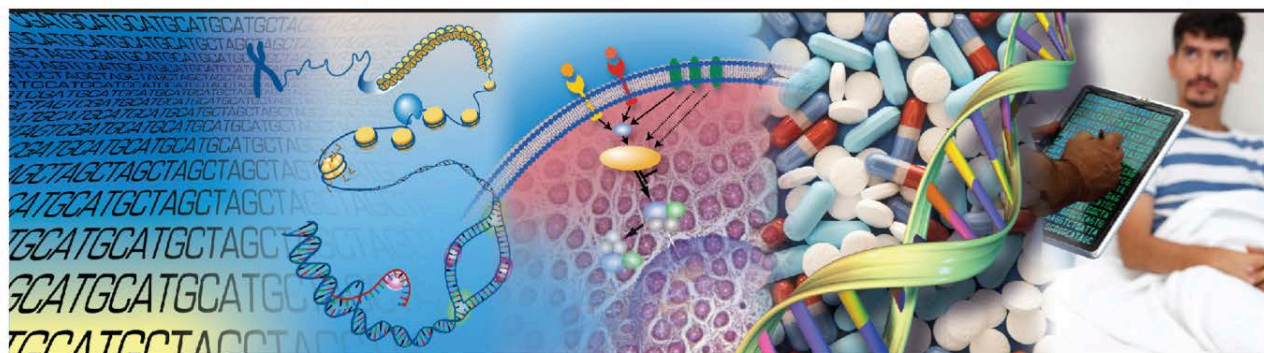
Understanding
the structure of
genomes

Understanding
the biology of
genomes

Understanding
the biology of
disease

Advancing
the science of
medicine

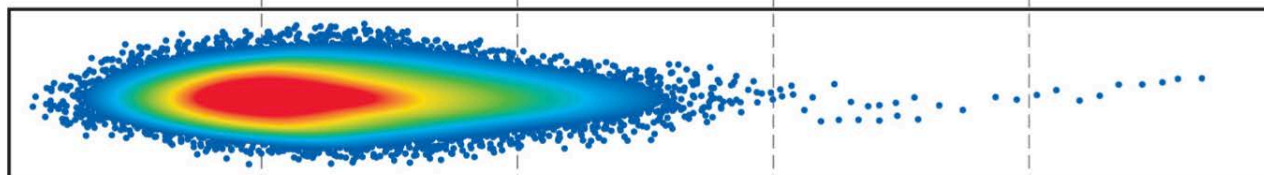
Improving the
effectiveness of
healthcare



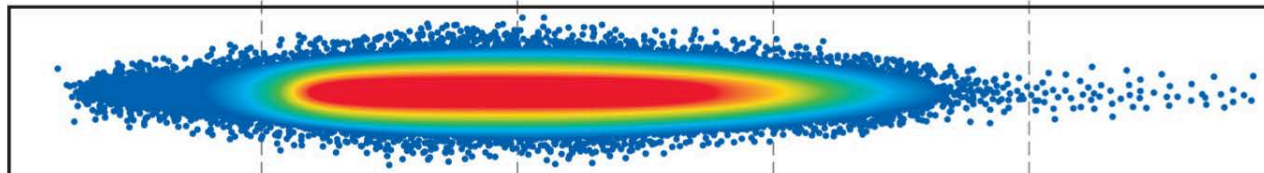
1990–2003
Human Genome Project



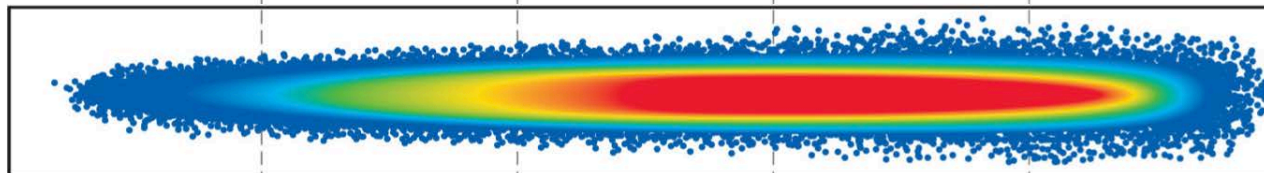
2004–2010



2011–2020



Beyond 2020



PERSPECTIVE

Charting a course for genomic medicine
from base pairs to bedside

Erik D. Green¹, Mark S. Guyer² & National Human Genome Research Institute^a

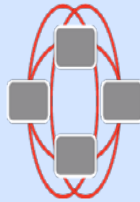
doi:10.1038/nature09704

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



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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

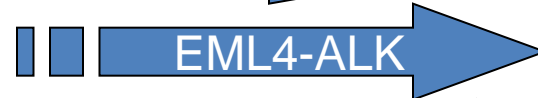




We Are Getting Faster!!!



Sle



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•www ... : What? – Why RAPM? - Why now?

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

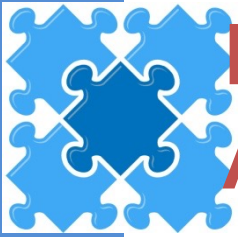
Why IAPM?

- 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

Clinical Evidence

Registeries

**Treatment decision-making
considerations**

Patient empowerment

**Collaborating with your
healthcare team**

Value/Cost Rubicon

**Research in Personalised
Medicine**

**Ensuring access for tests
AND treatment**

Bio-banking

Public Private Partnerships

**Expanding organizational reach to
untapped patients**

**Genetic, genomic and NGS
testing**

Definitions

Role of Ethics Committee

Educating policymakers

**Establishing
consistent language**

Big Data & Research





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: 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: 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.”



Stream 4: Medical Adaptive Pathways

Key Policy Asks:

- A) There is a need for a better understanding of patients’ and payers’ willingness to operate with greater uncertainty driven by the release of needed therapies with less evidence at the initial launch;
- B) There is a need to facilitate the IT infrastructure and processes to provide the necessary evidence base using real-world data

There are four thematic focuses in this Working Group:

Stream 1: Clinical Trials

Policy Ask: To ensure a responsive regulatory environment that responds to the needs of all stakeholders whilst ensuring patient safety, with the end result of ensuring development of treatments for patients

Stream 2: Data Protection

Policy Ask: easier circulation and sharing of personal data, appropriately balanced with personal privacy for the benefit of patients

Stream 3: In-Vitro Diagnostics

Policy Ask: greater attention to the role of diagnostics and imaging in personalised medicine

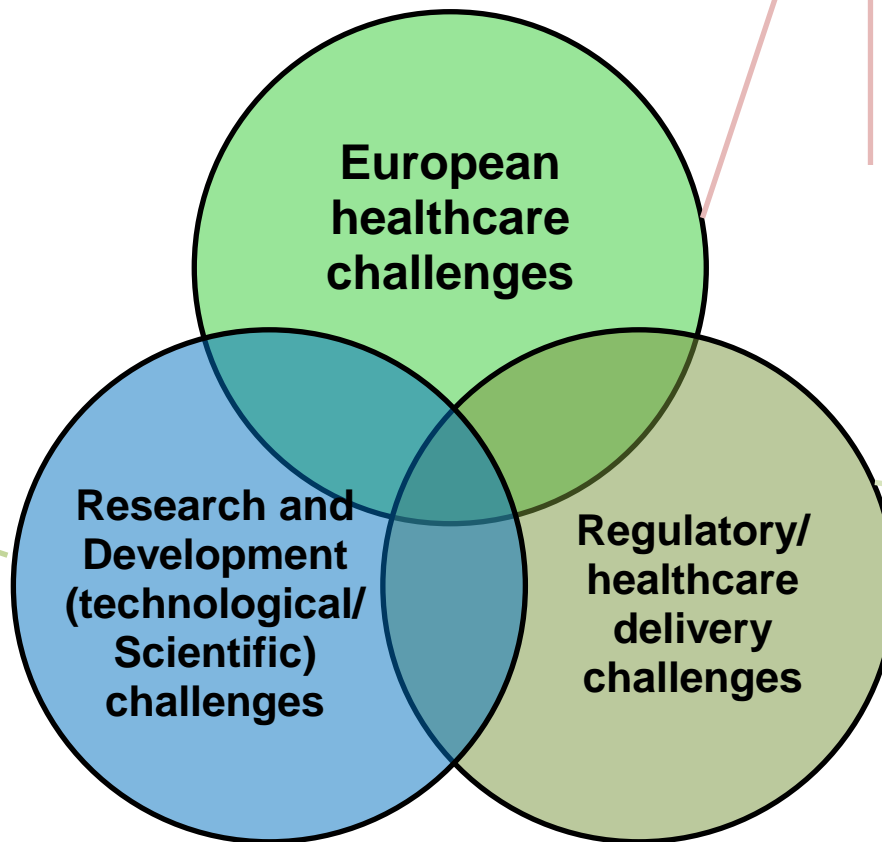
Chair: ROCHE & European Patient Forum

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



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

How does this impact R&D?

What solutions can be driven through R&D?

How does this impact healthcare delivery?

What research is required to provide solutions?



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DEFINITION OF PERSONALISED MEDICINE

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;



Brussels, 7 December 2015

(OR, en)

1556415

SAN 428

OUTCOME OF PROCEEDINGS

From: General Secretariat of the Council
On: 7 December 2015
To: Delegations
No. prev. doc.: 14363/15
Subject: Personalised medicine for patients
- Council conclusions (7 December 2015)

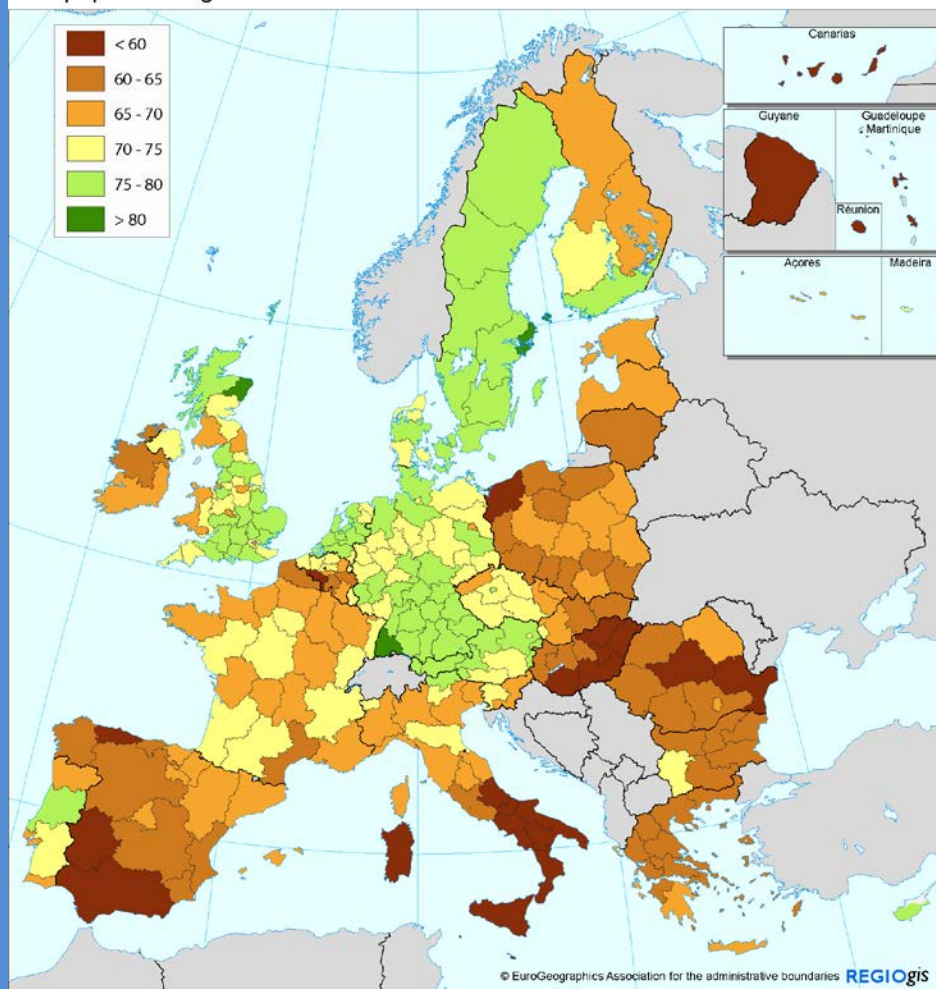
Delegations will find in the annex the Council conclusions on personalised medicine for patients, adopted by the Council at its 14348th meeting held on 7 December 2015.

Growing divide across Europe



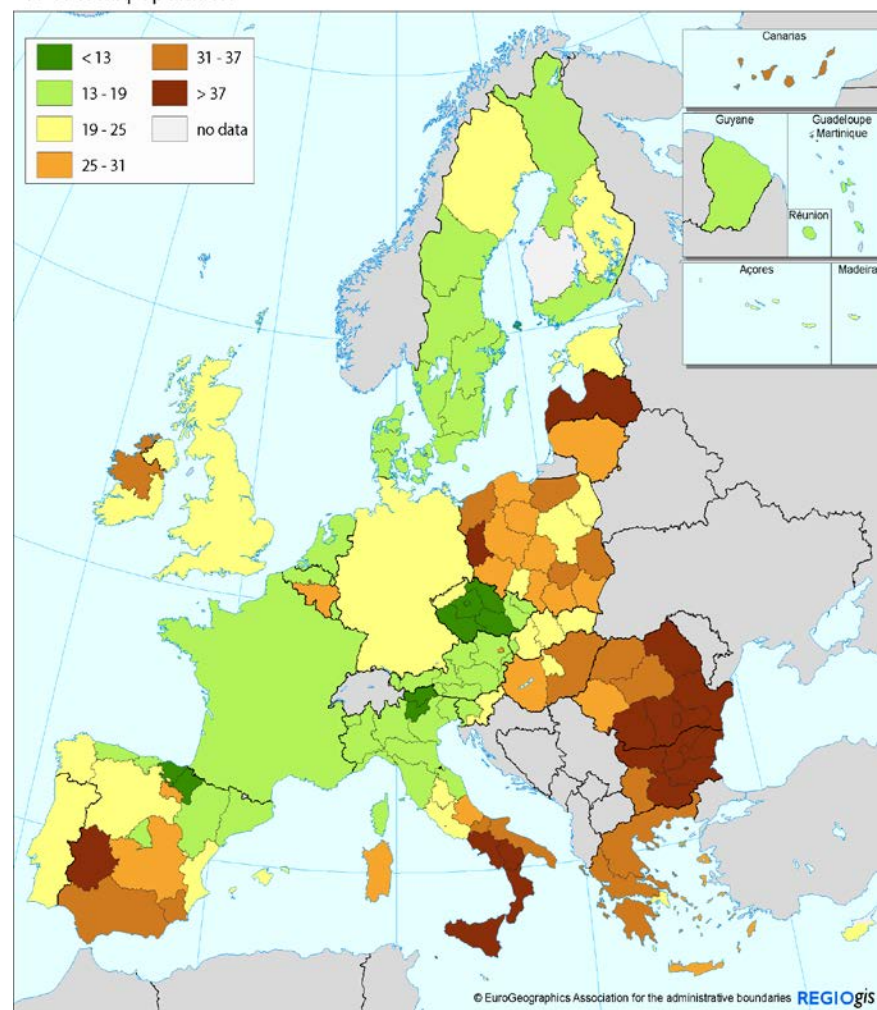
Employment rate, 20-64 in 2010

% of population aged 20-64



Population at risk of poverty or exclusion, 2009

% of total population



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“An imbalance between rich and poor is the oldest and most fatal ailment of all republics”

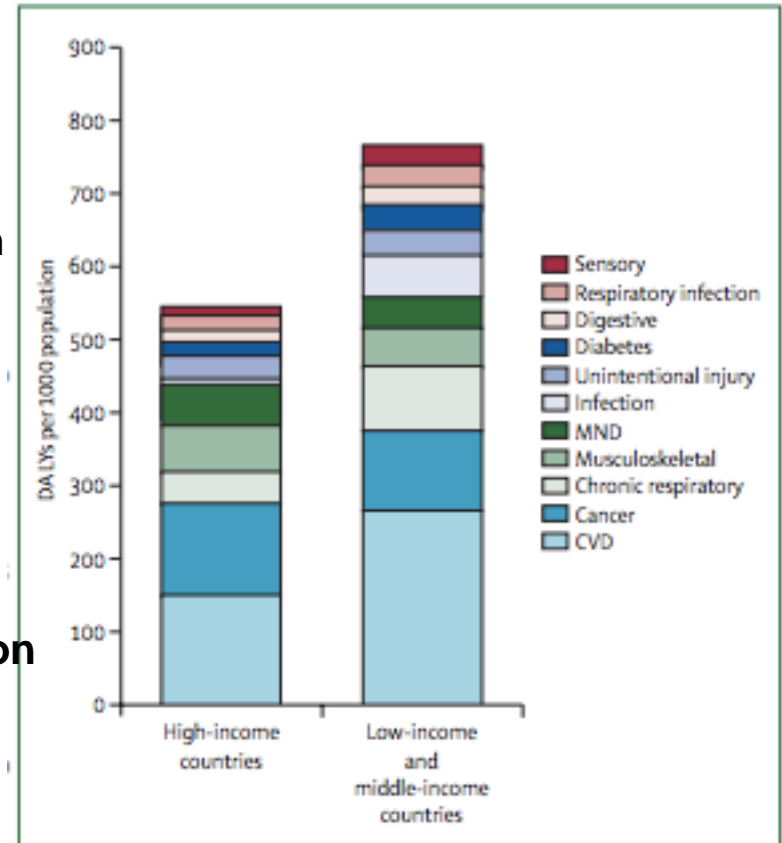
Disparities in male life expectancy between East and West Europe have grown in the last 40 years

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



Prince M, Sullivan R *et al.*

The burden of disease in older people: implications for health policy and practice

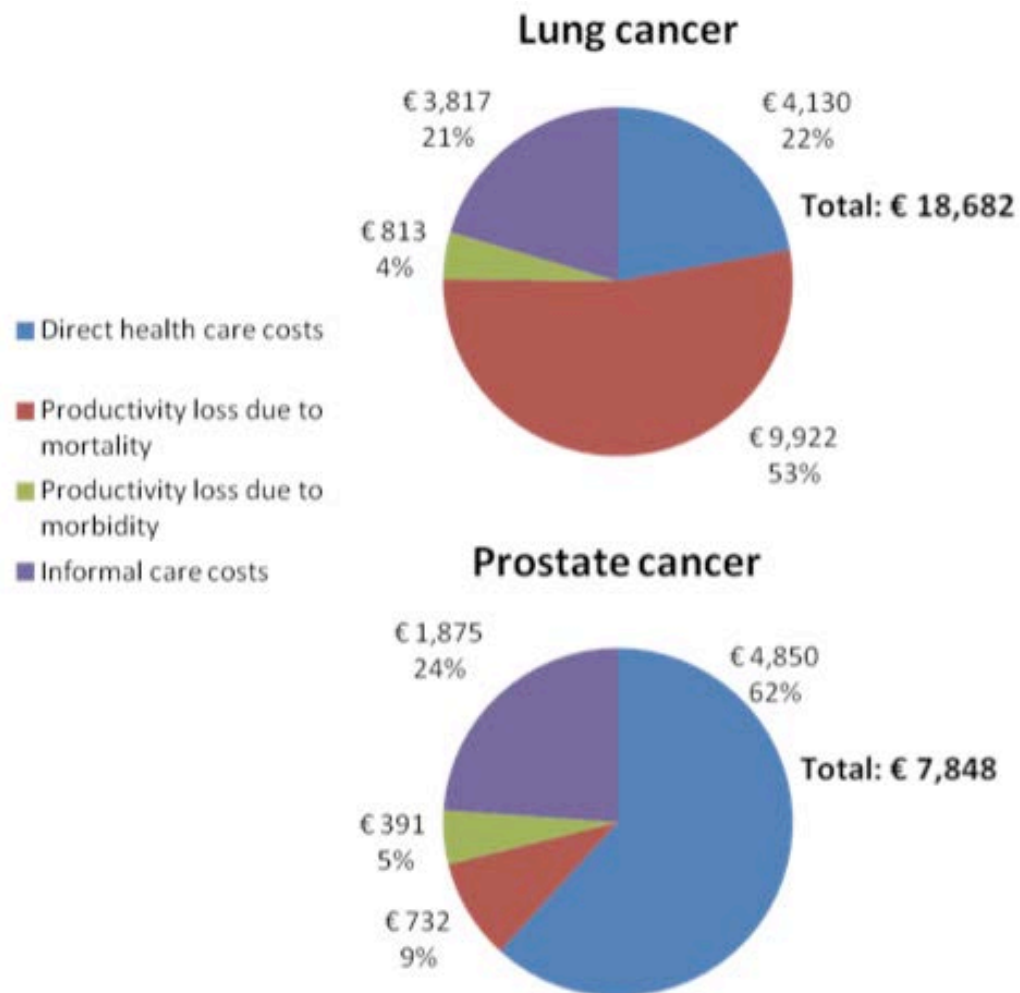
Lancet Series on Ageing Nov 6th 2014



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



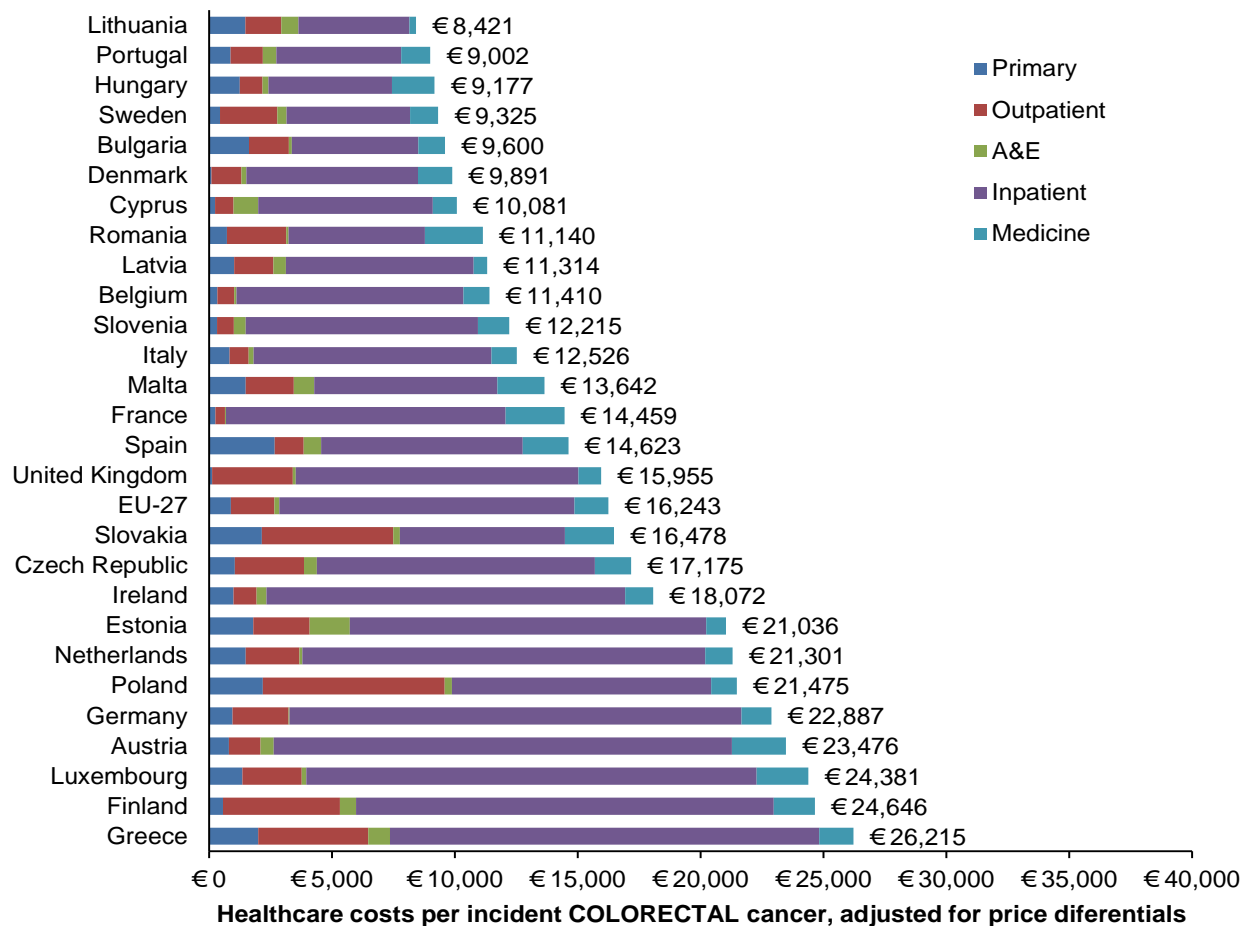
Fernandez-Luengo R, Leal J, Gray A, Sullivan R. **Economic burden of cancer in Europe.** Lancet Oncology 2013; 14(12): 1165-1174.



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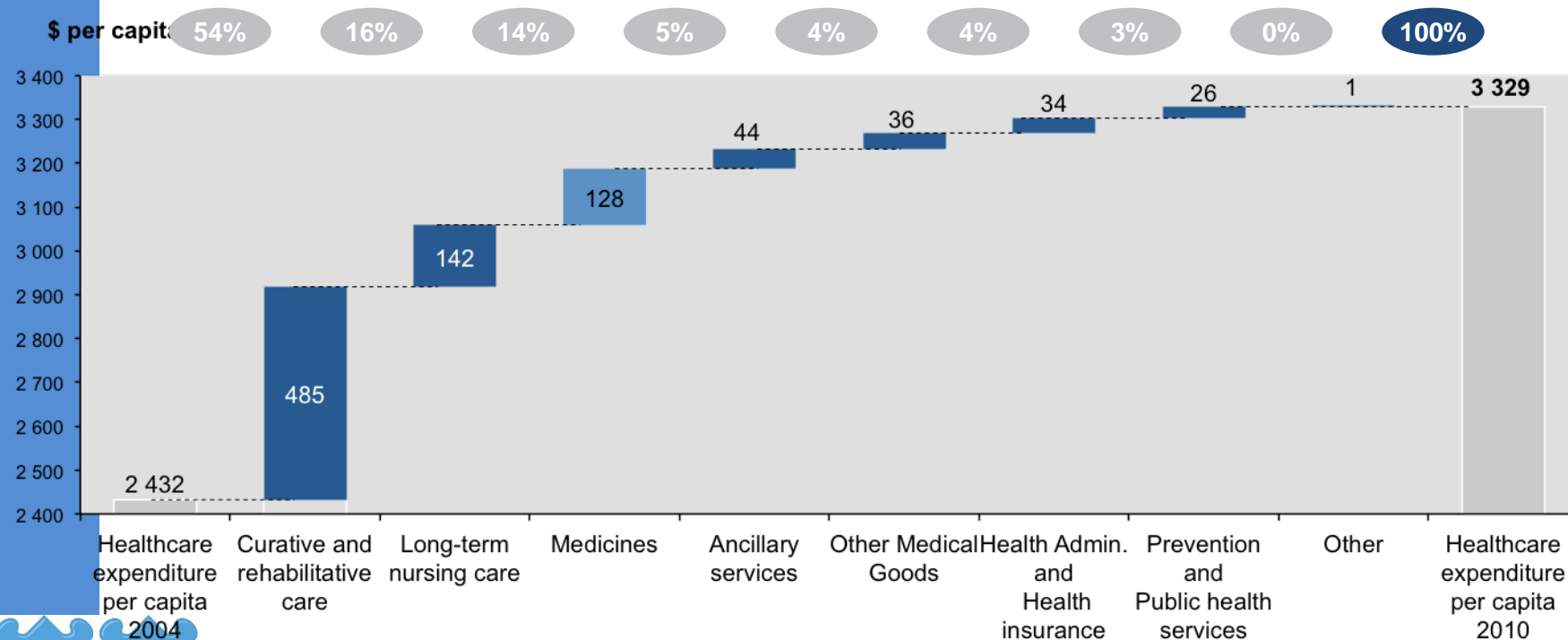
Massive variation in direct spend on cancer care across Europe: *major differences in where money is spent*





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



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Total cost 2 billion euro

The bridge over öresund between Sweden and denmark



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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 II





Council Conclusion on PM



Council of the
European Union

Brussels, 7 December 2015
(OR. en)

15054/15

SAN 428

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.

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



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What we got: 2016 Council Conclusions on PM

HCP
education

HTA, STAMP

Promote
H2020 results

Access to PM
therapies

Public
education

GDPR

Study on Big
Data for PM

Use genomics
for public
health

Specialists
collaboration

Data
standards,
collection,
sharing and
processing
inc biobanks
and EHRs

Leverage
ERNs for R&D

Best practices
& MS dialogue



Council of the
European Union

Brussels, 7 December 2015
(OR_en)

15054/15

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BUT, healthcare systems ...

- Function nationally
- Have national efficiency as their highest priority





Developing diagnostics for Personalised Medicine

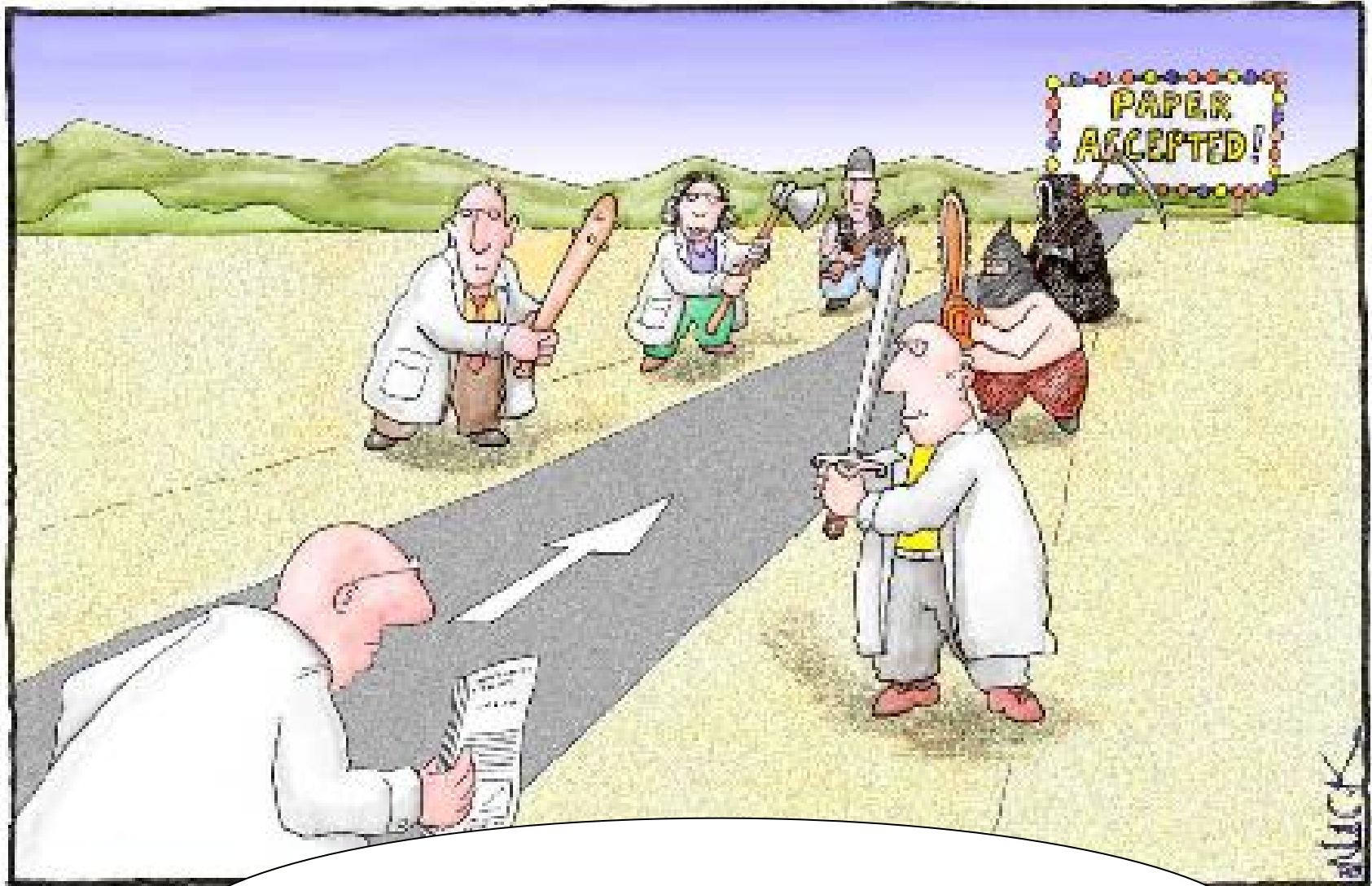
- What is the 'right' level of clinical evidence for a companion diagnostic?
 - Balance of scientific rigour and access to innovation
- Should lab developed tests have to meet the same criteria as other companion diagnostics?
 - If not, what Quality Assurance and audit measures should be in place?
 - 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



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Navigating the legislative Barriers

Interoperability – Health Systems

Um Gottes Willen, was hat er gesagt?

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?



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The political context

The whistleblower



I can't allow the US
government to
destroy privacy
and basic liberties

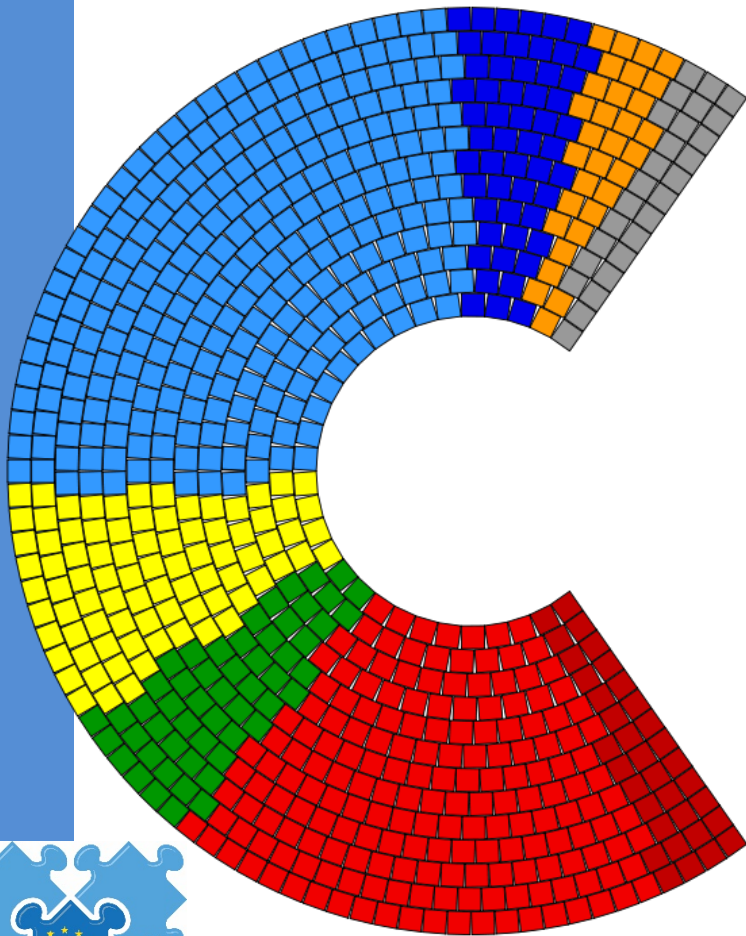
the guardian

guardian.co.uk





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 changing**¹
- Provide **real hope** for PM integration
- **BUT**
- **Fragmentation, Silo Mentality and other Barriers threaten its translation** into National Health Systems ^{2, 3}



¹ Lawler M., Selby PJ "Personalised Cancer Medicine: Are we there yet?" *Oncologist*. 2013; 18: 649-50

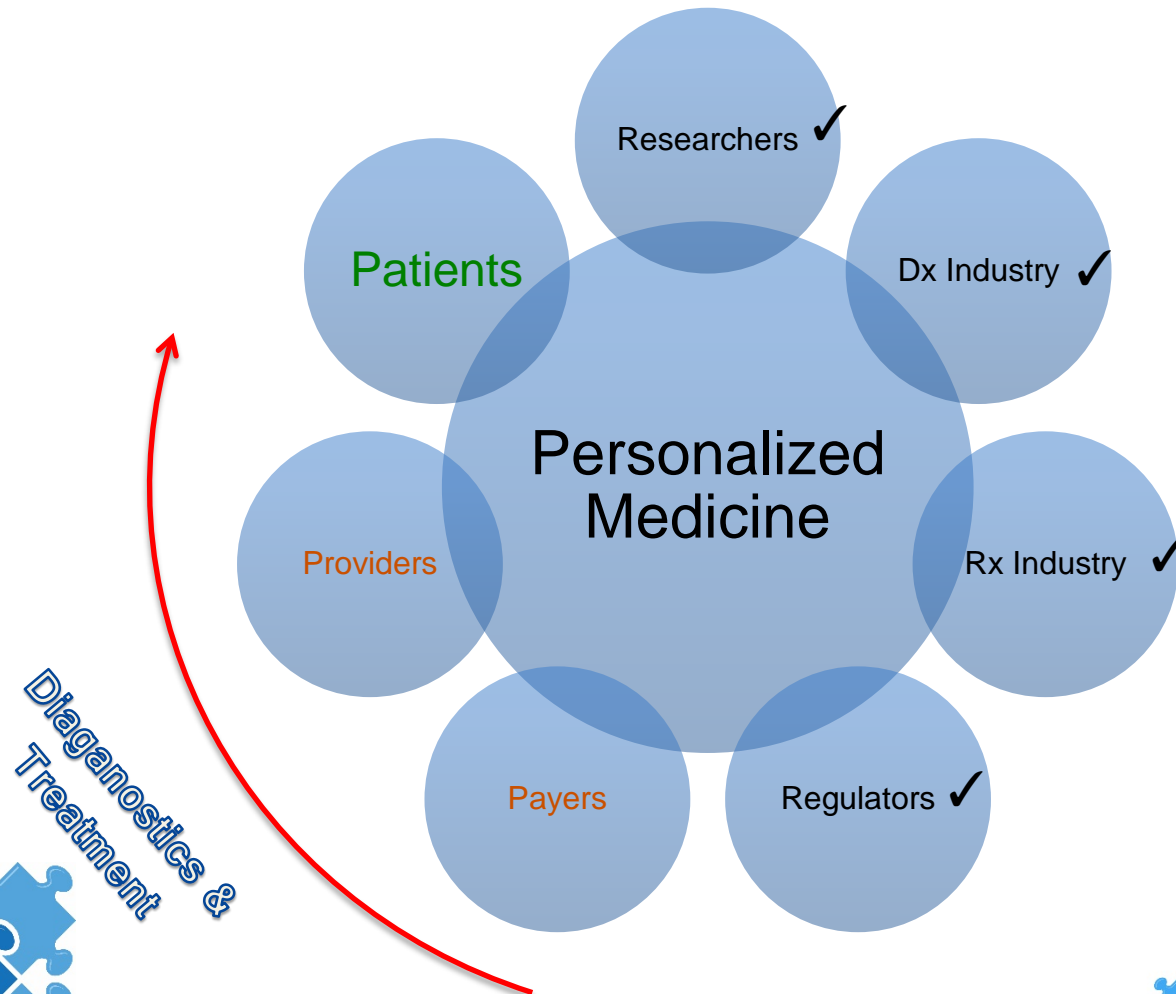
² "Barriers in Access to Personalised Medicine: Report on the development of an EU index." EAPM 2014

³ Use of 'omics technologies in the development of personalised medicine EC 2013





Key Stakeholders





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



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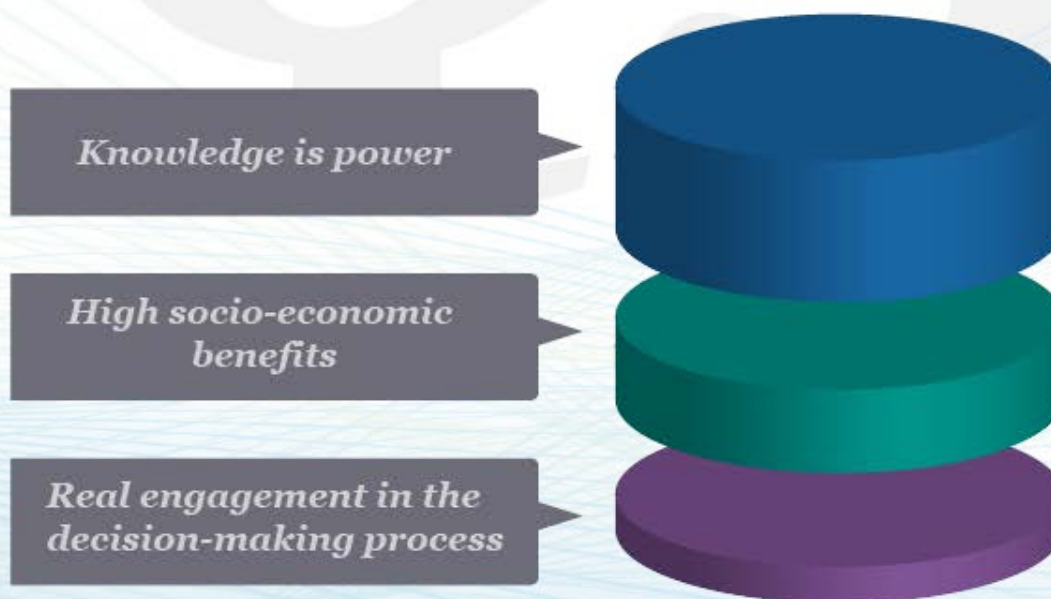


Q1 *How interested would you be in having a test done that would provide you / your doctor with information to guide therapy choices for cancers, infectious diseases, neurological disorders (e.g. Alzheimer's disease), and other conditions, while also avoiding unnecessary treatments with little chance of success and side effects?*



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Q5 *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



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Geographical scope

Italy
Poland
Spain
France
Germany
All-Ireland
United Kingdom
Bulgaria
Romania
Sweden

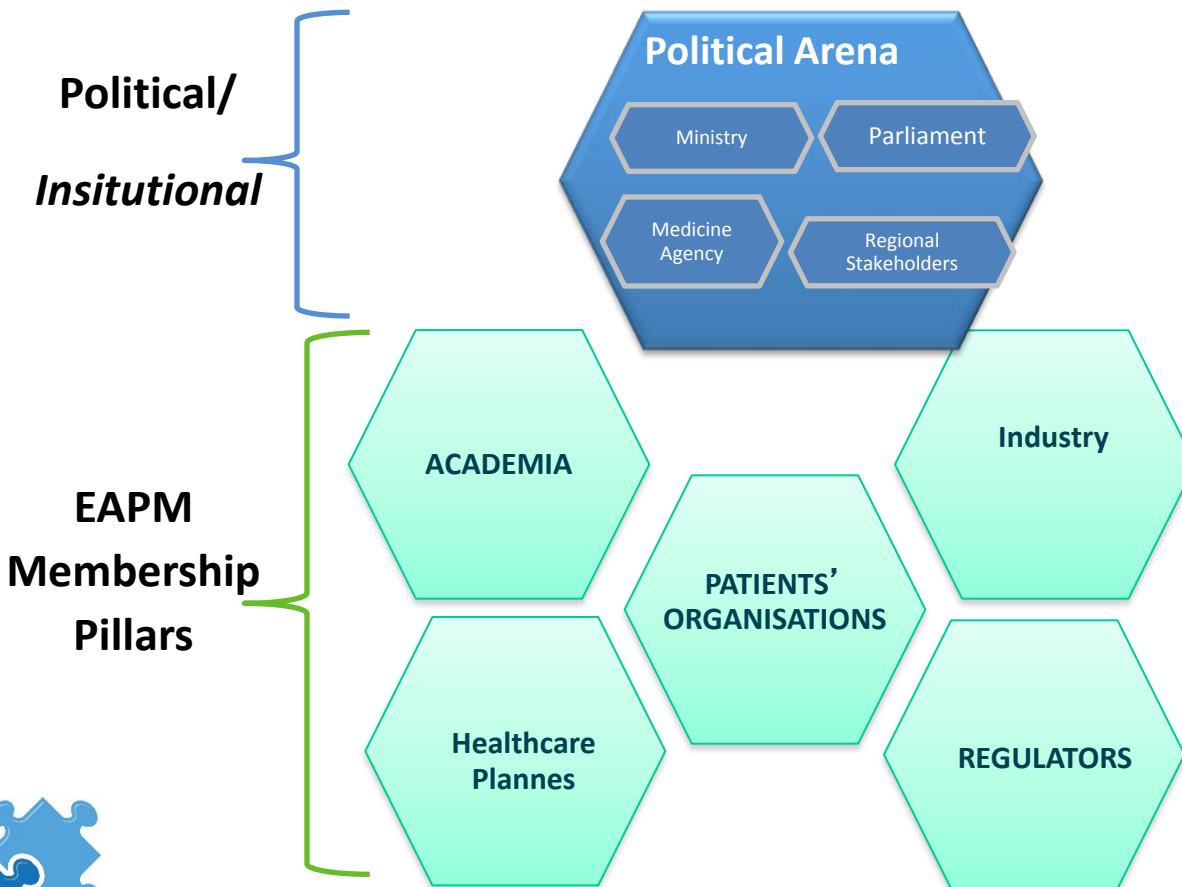




Romanian Alliance for Personalised Medicine

Stakeholders

Stakeholder Engagement- Communication



FUTURE OF PERSONALIZED MEDICINE



BETTER EVIDENCE
FOR DIAGNOSTICS
AND THERAPIES



NEED MORE
AGILE
REGULATORY SYSTEM

GENETIC COUNSEL
+
DECISION SUPPORT
+
GENETIC LITERACY

TRANSLATE RESEARCH...



...EMPOWER PATIENTS!

TAKE CARE OF
YOUR OWN HEALTH!



TEST BEFORE YOU
TREAT



GET TO THE
RIGHT DRUG



THE FIRST TIME!



ALL
OF THE DATA
FROM THE INTERNET
CAN BE STORED IN DNA
IN A SMALL TEST TUBE.

GIANT LEAPS IN
MEDICINE
ARE JUST AROUND
THE CORNER!



Thank you!

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