

Personalised Medicine

ESF Workshop on Health Research Strategic Needs in Europe
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1. “Can Europe lead the global way?”

2. Challenges, bottlenecks, gaps and needs?

1. “Can Europe lead the global way?”

European activities and milestones?



a. European Science Foundation (ESF)



ESF Forward Look

Personalised Medicine for the European Citizen

Towards more precise medicine for the diagnosis, treatment and prevention of disease (iPM)



ESF Position Paper

May 2011

Technology

19-20 Sept 2011
London, UK

- 1) CV & metabolic diseases
- 2) Oncology
- 3) Rare Diseases

Disease Summit

18-20 Oct 2011
The Hague, NL

"Big picture" Summit on clustered issues

13-14 Feb 2012
Dubrovnik, HR

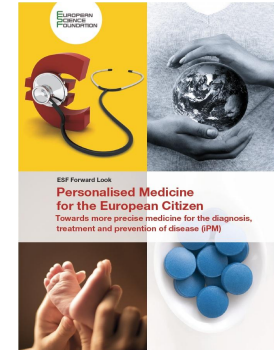
Identify grand challenges and recommendation

Stakeholder conference

18 April 2012
Rome, IT

Consensus discussion on Grand Challenges and overall recommendations

b. CSA-PerMed



FP7 CSA - **PerMed**

Personalized Medicine 2020 and Beyond – Preparing Europe for Leading the Global Way

Consortium: **Research and Health Ministries (funding bodies)**

Connected to other key European initiatives in Personalized Medicine (e.g. ESF, PHGEN, EHFG, EAPM, EPEMED, HOPE)

Aim: Strategic Research and Innovation Agenda (SRIA) for Europe

1st Workshop of stakeholders March 27/28th 2014 in Berlin

Parallel Forum at the European Health Forum Gastein (EHFG), October 1-3, 2014

Discussion SRIA & PerMed Roundtable, Barcelona, March 25-26, 2015

Webpage: <http://www.permed2020.eu>

c. European Health Forum Gastein (EHFG)

EHFG 2006, 2010, 2011, 2012, 2013 and 2014





European Alliance for
Personalised Medicine




BIG DATA
Workshop
16th European Health Forum Gastein
REPORT - OCTOBER 2013



EUROPEAN COMMISSION
Research & Innovation



d. European Alliance for Personalised Medicine (EAPM)

Specialised Treatment for Europe's Patients – „STEPS“

informed about, and engaged in, their own healthcare. Regulators must make it possible to allow patients early access to novel and efficient treatment.

Together we can transform our healthcare system into one that delivers the best care for patients, empowers them, puts research and innovation at their service, and gives the best possible value. But the right conditions must be in place.

Empowering patients depends upon each being treated as an individual, taking into account not only the illness but also differing social and cultural backgrounds.

The patient needs to be involved in every aspect of treatment. Education and simplified, standardised information, plus full access to all relevant medical files, are cornerstones of this. The patient should have access to all possible treatments.

With the support of the European Commission and Parliament we can make this happen. It is the responsibility of all of us and all stakeholders must devise an aligned strategic research agenda, with input from all disciplines but including the patients.

All EAPM members (patients, medical professionals, healthcare planners, industry, scientists and researchers) share the vision of a Europe which inspires trust in its health systems. Unnecessary deaths of patients could be avoided via greater access to innovative treatment and diagnostics and efficiently organised research.

With this in mind, the European Alliance for

- **STEP 1: Ensuring a regulatory environment which allows early patient access to novel and efficacious personalised medicine (PM)**

- **STEP 2: Increasing R&D for PM, while also recognising its value**

- **STEP 3: Improving the education and training of healthcare professionals**

- **STEP 4: Supporting new approaches to reimbursement and HTA, required for patient access to PM**

- **STEP 5: Increasing awareness and understanding of PM**

EAPM believes that achieving these goals will improve the quality of life for patients in every country in Europe.



European Alliance for Personalised Medicine

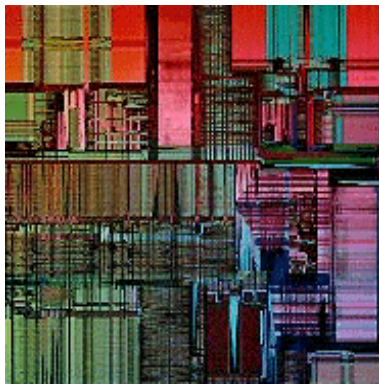
An introduction to EAPM, its Policy Taskforces and STEPs campaign



2. Challenges, bottlenecks, gaps and needs?

(Public) Health moves towards Personalised Medicine ...

(Public) Health



Genomics



Big Data



Public Health Genomics (PHG) – Personal Health and Care



The BIG4HEALTH 😊
- four key research policy areas for Europe ...

1. decision-supporting tools

2. “big data”

3. ownership

4. health systems

1. decision-supporting tools

- **HTA 3.0** (assumption non-linearity and “personal evidence”)
- systematic early dialogue/**PPP** (e.g. LAL model, **MAPPs**), best practice of **PPP = IMI**
- “just in time” interventions (JITs)
- orphan drug model & pilots (e.g. Germany: “Heilversuch” with N=25) / RoI
- drug/theranostics/CDx/IVD versus Medical Device ... (use of) health information (HI)
- “virtual twin”: in silico “try and error” (simulations, artificial learning)

2. “big data”

- **N=1 trials:** “I am my own reference point”
- **N=all trials:** mission impossible (“big data” will always be incomplete)
- **unstructured** (and structured) data for **unknown future purposes** (more than just data linkage or open access)
- **validation, standardization:** mission impossible (always a “momentum”)
- **“incidental findings”/noise:** all findings are important, we just cannot interpret them (yet): “junk versus garbage”
- **health information will always be “messy”/chaotic: what (not why) is good enough** in most cases! **Correlation** versus causality ...

3. Ownership

- “I am the owner of my data”: personal ownership (property based, excluding right, paternalistic) vs. **citizen ownership/control** (broader, social right, shared right, democratic)
- from informed consent (blanket or broad) & privacy issues to data-users accountability: “trust & trusts”! (... to guarantee data security is dishonest!)
- “big data” meet governance of information via algorithm providers (QM): rules of impartiality, confidentiality, competence (interpretation of data) and professionalism
- **Health Data Cooperatives** (balance between public good – personal benefit, no monetary incentives for individuals!)

4. health systems

- **“good governance”** – “good” implementation of “good” health policies (e.g. in Europe cross-border directive, **“bottom-up” policies**)
- WHO-EU Regional office (Tallinn, 2008) : **six system building blocks**



The next step???

ERA-net on Personalised Medicine!



DANKSCHEEN
 GRACIAS
 SHUKURIA
 JUSPAKAR
 GOZAIMASHITA
 EFCHARISTO
 KOMPA-SUNNIDA
 MAANE
 GRAZIE
 MEHRBANI
 PALDIEN
 BOLZIN
 MERCI
 TASHAKKUR ATU
 YACHANYELAY
 SUKSAMA
 EKHMET
 SHUKRIA
 BIYA
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 TINGKI
 YOU HANK

