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Personalised Healthcare: Policy and Practice progress

FEAM Conference 2018 on Precision Medicine and Personalized Health Friday,

28 September 2018
Campus Biotech, Geneva, Switzerland Hosted by the Swiss Academy of Medical Sciences

Denis Horgan, EAPM Executive Director

Personalised medicine: building a bridge to future

European Council conclusions on Personalised Medicine for patients

No commonly agreed definition of the term "personalised medicine".

<u>Widely understood that personalised medicine refers to</u> 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**

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- **….Key objectives….**

Support **citizen-focussed innovation** and contribute to a vibrant lifescience sector in Europe

Goal: facilitate patients' access to innovative medicines

- Optimise the use of existing regulatory tools including:
  - Monitor the progress of adaptive pathway project
  - Identify the challenges along the lifecycle of a personalised medicinal product
- Expert group on safe and timely access of patients to medicinal products (STAMP)
- **Study Big Data***
- **Cooperation on HTA***
- Commission expert groups
- European Reference Network

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Key drivers – Bringing innovation into healthcare systems

Drivers

- EU countries face similar challenges
- Need an integrated approach
- Increased focus on health outcomes
- Need to empower citizens and patients
- Economics - ‘necessity is the mother of invention’

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A diversified palette of instruments for R&I -1-

- **More than 1,000 transnational collaborative projects underway, with over 10,000 participants.**

  → Increased European human capital on health research and its excellence. Networking of health R&I across Europe and beyond.

- **A public-private partnership** (the world's largest in health): IMI

  → European-wide unmet medical needs addressed; building on the complementary strengths of industry and academia.

- **Coordinating national funding programmes** : EDCTP, JPND, JPAMR, HBM4EU

  → EU helps countries pooling their resources.

- A projected 56,000 high-level scientific articles on health

- 500,000 new compound candidates for new drugs made available to other researchers; and experts across Europe (IMI)

Illustrative example from Horizon 2020: tackling the global challenge of rare diseases

- *7 % of the world's population*
- *24 % of global GDP, it produces around*
- *30 % of the world's scientific*

"Europe suffers growth deficit

- *uneven progress,*
- *fuels social disenchantment and*
- *political divisions across the continent.*

Investment of R & I

- An increase in R&I investment of **0.2% of GDP** would results in an increase of **1.1% GDP** in productivity growth
- Private returns 10% - 30%

6,000-8,000 diseases - more than 30 million patients in EU

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Health Technology Assessment (HTA)

Proposal for a
Regulation
31 January 2018

WHAT'S NEW?

- **Common** European **assessment methods**
- **Shared data** and expertise
- **Common procedures** across the EU

WHAT ARE THE BENEFITS

- Higher level of **human health protection**
- Faster market access for **innovative products**
- More **transparency** for patients and producers
- No more **duplication** of work for health authorities and industry

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EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT

NEW MEDICINES

EU ASSESSMENT (jointly done by the Member States)

NATIONAL ASSESSMENT

National decisions on pricing and reimbursement

CLINICAL ASSESSMENT (benefits compared to existing treatments)

NON-CLINICAL ASSESSMENT (economic, social and ethical aspects)

NEW MEDICAL DEVICES

High-risk devices with high impact on patients, public health and EU health systems

CLINICAL ASSESSMENT (benefits compared to existing treatments)

NON-CLINICAL ASSESSMENT (economic, social and ethical aspects)

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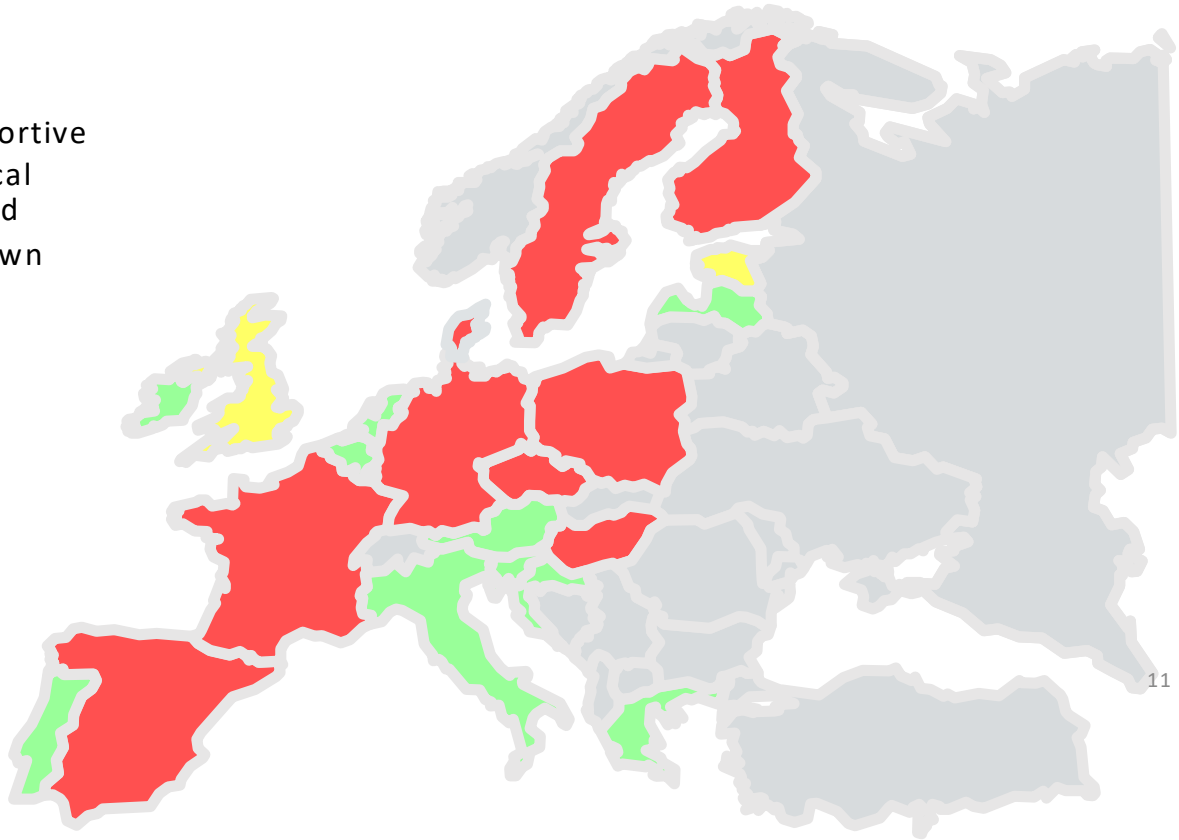
Member States' positions - graphical overview of the HTA

- Green : supportive
- Yellow : critical
- Red : opposed
- Grey : unknown



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"Drug innovation has also transformed the treatment of grievous illnesses, such as cancer - where we have seen significant value delivered at societal and individual patient level, with the promise of more to come"

- **Experts estimate that innovative cancer drugs introduced between 1975 and 1995 have:**
  - Accounted for **~ 50-60% of the increase in age-adjusted survival rates in the first 6 years after diagnosis**
  - **Added > 1 year of life to patients diagnosed with cancer in 1995**
  - Increased the life expectancy of the *entire U.S. population* by 0.4 years (since lifetime risk of being diagnosed with cancer is ~ 40%)
  - Recent research suggests the value to the patient of a life year saved **in cancer is actually closer to ~$300K, well above the typical $30-75K QALY values used in health economics**

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The Translational Challenge

- There is a **wide research-to-market gap in Europe**. The latest EU innovation scoreboard, published in June 2017, noted that although the innovation performance of the EU is improving, **progress is too slow**. Many of our global competitors are increasing their innovation performance at a faster pace, and **performance gaps remain wide within the EU** itself. **Europe's comparative advantages in education, research, broadband infrastructure and ICT training are not matched by venture capital investments and the number of SMEs introducing innovations, both of which are declining strongly.**
- Europe still faces harsh choices about **whether it is actually going to do what it has so often discussed.**

Extracts from Horgan et a, IBiomed Hub 2017;2(suppl 1):481130

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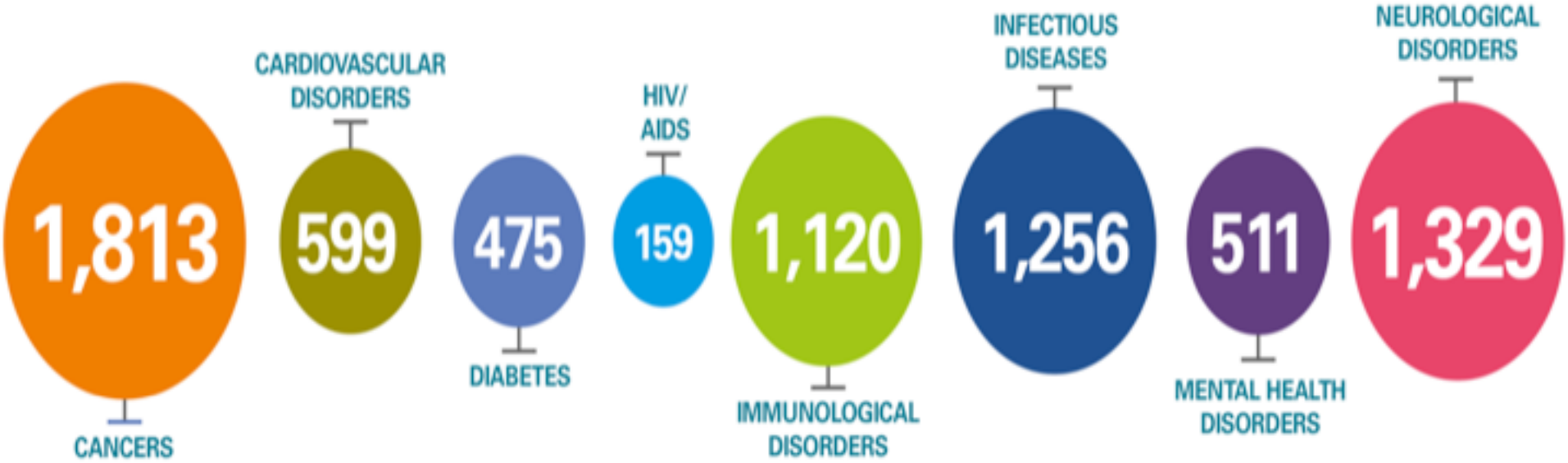
What is Innovation ?

Innovation = translation of knowledge and insight to <u>value</u>

Value=

- **Value to patients**
- **Value to Society**
- **Return on Invetsment**

With over 7000 medicines in development, the exciting new wave of medical innovation will play a key role in **addressing the challenges faced by patients and healthcare systems**



Source: Health Advances analysis; Adis R&D Insight Database. March 2015, compiled by PhRMA

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Juncker guidelines

- **Priority 1**: A New Boost for Jobs, Growth and Investment
- **Priority 2**: A Connected Digital Single Market
- **Priority 3**: A Resilient Energy Union with a Forward Looking Climate Change Policy
- **Priority 4**: A Deeper and Fairer Internal Market with a Strengthened Industrial Base
- **Priority 5**: A Deeper and Fairer Economic and Monetary Union
- **Priority 6**: A Reasonable and Balanced Free Trade Agreement with the USA
- **Priority 7**: An Area of Justice and Fundamental Rights Based on Mutual Trust
- **Priority 8**: Towards a New Policy on Migration
- **Priority 9**: A Stronger Global Actor
- **Priority 10**: A Union of Democratic Change

- "**Lamy report**" - Recommendation 5: Adopt a mission-oriented, impact-focused approach to address global challenges.

  Action: Set research and innovation missions that address global challenges and mobilise researchers, innovators and other stakeholders to realise them.

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Criteria for selecting R&I missions

proposed by Prof Mazzucato

- Bold, inspirational, with wide societal relevance
- A clear direction: targeted, measurable and time-bound
- Ambitious but realistic R&I actions
- Cross-disciplinary, cross-sectoral and cross-actor innovation
- Multiple bottom-up solutions

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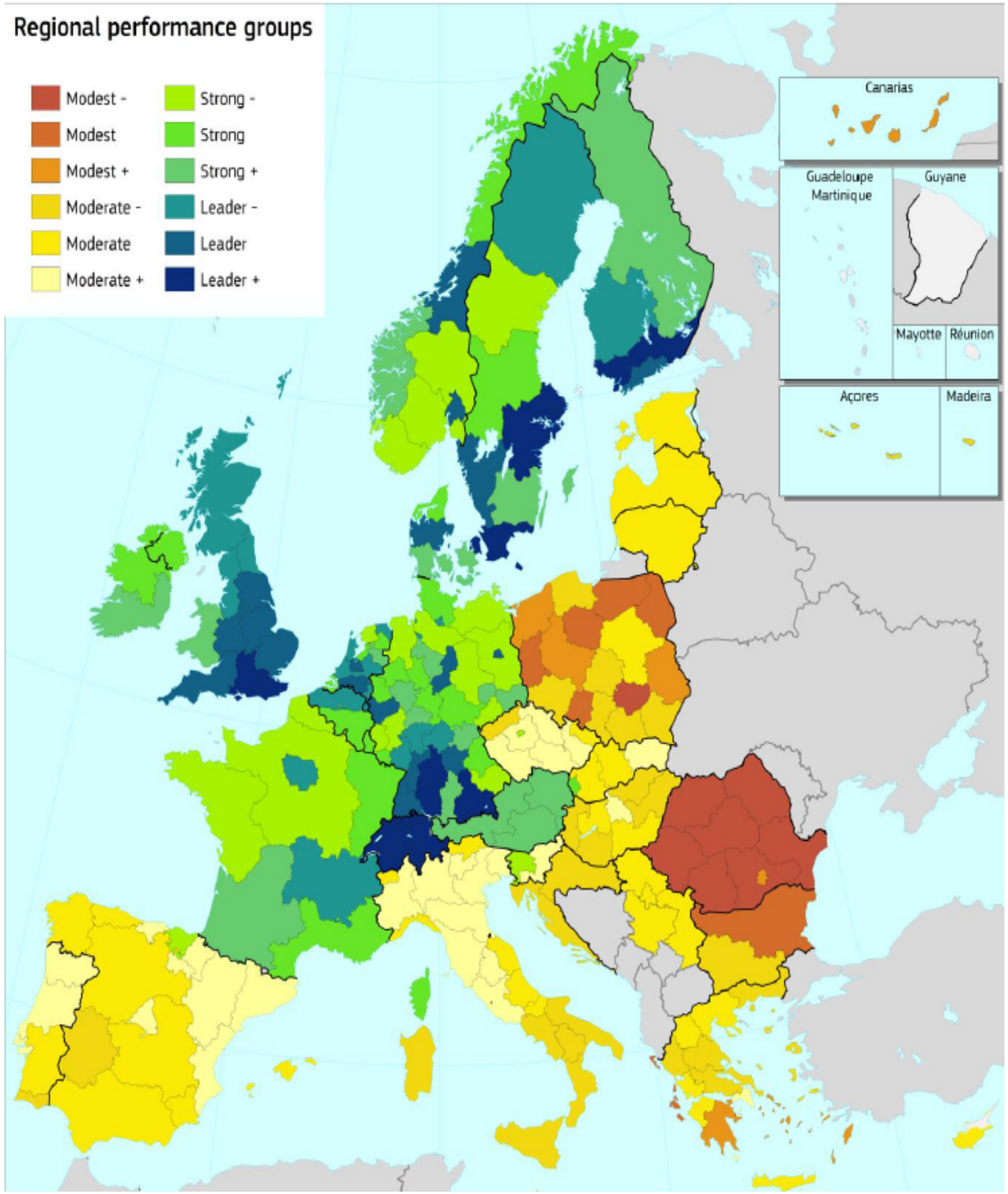

Regional Innovation Scoreboard 2017

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In 2018, the European Commission initiated a **formal process of reflection on options**:

Five scenarios were:

1. Carrying on much as at present, with the EU27 focusing on "its positive reform agenda"
2. Nothing but the Single Market, with the EU27 "gradually re-centred on the single market"
3. Those Who Want More Do More: The EU27 allows willing Member States to do more together in specific areas
4. Doing Less More Efficiently: The EU27 focuses on delivering more and faster in selected policy areas, while doing less elsewhere
5. Doing Much More Together: Member States decide to do much more together across all policy areas

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What challenges lie ahead ?

1. Unsustainable health and care costs	2. Role of environment on health	3. Increasing risks of infectious diseases	4. Health inequalities and access to health and care	5. Keeping a competitive European health and care industry
				
Increasing prevalence of chronic diseases and an ageing population	Lifestyle, pollutants, climate change	Resistance to antibiotics, (re)-emerging diseases	Access to health promotion, prevention, treatment	Keep Europe as the best innovator in healthcare area

- **Health care expenditure of EU countries is steadily increasing and now accounts for nearly 10% of GDP**, as a result of increasing prevalence of chronic diseases due to unhealthy lifestyles and an ageing population.
- Avoidable environmental factors cause 1.4 million deaths yearly in the EU.
- Infections resistant to antimicrobials (AMR) cause **25'000 deaths yearly in the EU and 700 000 deaths globally.**
- Life expectancy in some **EU countries is six years lower than the average.**
- EU healthcare industry accounts for **3% of GDP and 1.5 million employees**.

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Action is needed in a hundred areas.
The need is often recognised.

But **action does not always** follow.

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Large and positive economic impacts

- Growth: **€50bn led to €500bn** estimated effects from innovations, new techs/ products
- Jobs: 130000 direct (10 yy) and 160000 indirect (25 yy)

Better conditions for business R&I investments

- Improved scientific excellence and skills
- Leveraged industrial participation
- Improved knowledge flows and networks
- Large research infrastructures

Addressed societal challenges

- Supported the creation of new tech markets
- Boosted SME's to do groundbreaking innovation
- Enabled impactful public-private partnerships

Moving from broad challenges to specific missions

"The 'granularity' of European research and innovation missions sits between broad challenges and concrete projects. Missions set clear and ambitious objectives that can only be achieved by a portfolio of research and innovation projects and supportive measures." (Mazzucato report)

Political Agenda Setting and Civic Engagement

Clear Targeted Missions

Portfolio of projects and bottom-up experimentation

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BIG DATA: areas of Regional/Global Health collaborations

- **define** what “Big data” and subsets means from a regulatory science perspective
- **share information** on the current and potential applications of “Big data” in society in order to be abreast with societal environmental change
- **prepare** for future developments (scientific/IT/organisational) to pave the way for an integrated approach to the way information/data is being **generated, gathered and used**
- Secure **expertise** within the regulatory networks leveraging excellence with existing scientific infrastructures
- **ensure progress** in those areas where regulators are already active or action is planned
- **Identify case studies for use of Big Data for health at regional and international level**
- Analyse and initiate discussion on **mid- and long- term objectives**

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Three Areas

Research

Clinical Applicability

Innovation Incubator

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Interoperability of digital solutions: a challenge for Big Data

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The Vision of MEGA

The vision of MEGA is to leverage the shared knowledge and resources from sequencing one million genomes catalogued other patient data to discover and develop new personalised therapies and diagnostics to benefit patients globally

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Member States signed – States Signed

- **Italy**
- Croatia, Bulgaria, Lithuania
- Czech Republic Luxembourg, ,
- Estonia, **Spain**, Malta, Sweden, Slovenia, Greece, Cyprus, UK, Finland, Portugal

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"Towards access to 1 million Genomes in the EU by 2022"

Joint Declaration indicating political support for linking* existing and future genomic databanks (on a voluntary basis) in order to reach a cohort of 1 million sequenced genomes accessible in theEU by 2022. This joint commitment will make it possible to:

- **Bring together fragmented infrastructure and expertise supporting** a shared and tangible goal (1 million sequenced genomes accessible in the EU by 2022),
- **Leverage and maximise** the investments already made by Member States at national and EU level, particularly in sequencing, biobanking and data infrastructure,
- **Reaching a larger cohort** that will provide sufficient scale to inform the significance of "signals" identified in genomic and associated data, leading to new clinically impactful associations.

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What is MEGA (Million European Genomes Alliance)?

MEGA is a multi-stakeholder public/private alliance recognised by participating Member States to coordinate and place this initiative on a realisable path to achieve the following objectives:

- Bring together fragmented infrastructure and expertise supporting a shared and tangible goal (1 million sequenced genomes accessible in the EU by 2022),
- Leverage and maximise the investments already made by Member States at national and EU level, particularly in sequencing, biobanking and data infrastructure,
- Reaching a larger cohort that will provide sufficient scale to inform the significance of "signals" identified in genomic and associated data, leading to new clinically impactful associations.

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Implementation of MEGA Requires:

Active involvement of these stakeholders collaborating closely

1. Patient groups
2. European Parliament
3. Medical associations
4. Pharmaceutical companies
5. Ministries of Health
6. Medical centres
7. Diagnostic Companies,
8. Hospitals
9. Information Technology Companies

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Many European National Strategies aligned on approach and benefits

- Integrate genomic data in to electronic health records and use this to support routine clinical decisions and an evidence-based and sustainable health system;
- Engage citizens by supporting their engagement in the use of genomic data for their own health and ability to contribute to research for societal benefit;
- Foster collaborative multi-disciplinary research in a hybrid research/care model to address the areas of most clinical need and ensure system adoption;
- Support a precision medicine industry encompassing development of genomics, health and technology enterprises in each country as well as encourage investment of leading global pharma, biotechnology and technology companies

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Core Enablers defined Across European National Plans (1 of 2)

- Citizen/patient-centred approach – developing the vision alongside the community and supporting them in contributing to the development of personalised medicine;
- Ethical principles and supportive legislation – ensuring a sound moral and legal framework for the challenges posed by genomics in a hybrid clinical, research, industry setting;
- Novel partnership models for academic and commercial innovation – bringing academia, health systems, companies and other stakeholders in partnership to efficiently use data and expertise for maximal gain;

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Core Enablers defined Across European National Plans (2 of 2)

- Access to genome sequencing technology – providing equitable access to technology and ensuring ensure consistent quality of information generated through adherence and development of standards and distribution of best practice;
- Capable information systems – establishing a platform for the secure access to data, and host an ecosystem of established and in-development tools to support the interpretation of this;
- Educated and engaged healthcare workforce – helping to address the human barrier to successful implementation of genomics and personalised medicine in healthcare.

The key tenets of MEGA

This initiative is patient-centred and embraces a collaborative public-private-third sector approach

- Shared value creation
- Respect for each other's commercial interests
- Ensure undistorted competition
- Transparency
- Minimised data lock-in

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MEGA will achieve these objectives by taking the following actions:

- Leverage and maximally utilise existing resources
- Develop a federated ‘knowledgebase’ of genomic and health information
- Surround these knowledgebases with platforms for clinical discussions, training, research, innovation & enterprise
- Establish a cross-border network of expertise
- Create a platform for participant engagement

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MEGA Governance: Multi-stakeholder committees

1. **Leadership and operations**
   - 1.1 Executive
   - 1.2 Programme management
2. **Clinical and Scientific**
   - 2.1 Consent and research governance
   - 2.2 Research
   - 2.3 Clinical and evidence-based implementation
   - 2.4 Clinical innovation and drug development
3. **Ethical Legal and Social Implications**
   - 3.1 Ethics
   - 3.2 Legal and Regulatory
4. **Education, Engagement and Communications**
   - 4.1 Public and patient engagement
   - 4.2 Research engagement
   - 4.3 Clinical education
5. **Data and Informatics**
   - 5.1 IT architecture and network
   - 5.2 Data security
   - 5.3 Bioinformatics
   - 5.4 Information governance
6. **Sequencing**
   - 6.1 Sequencing infrastructure
   - 6.2 Protocols and Standards

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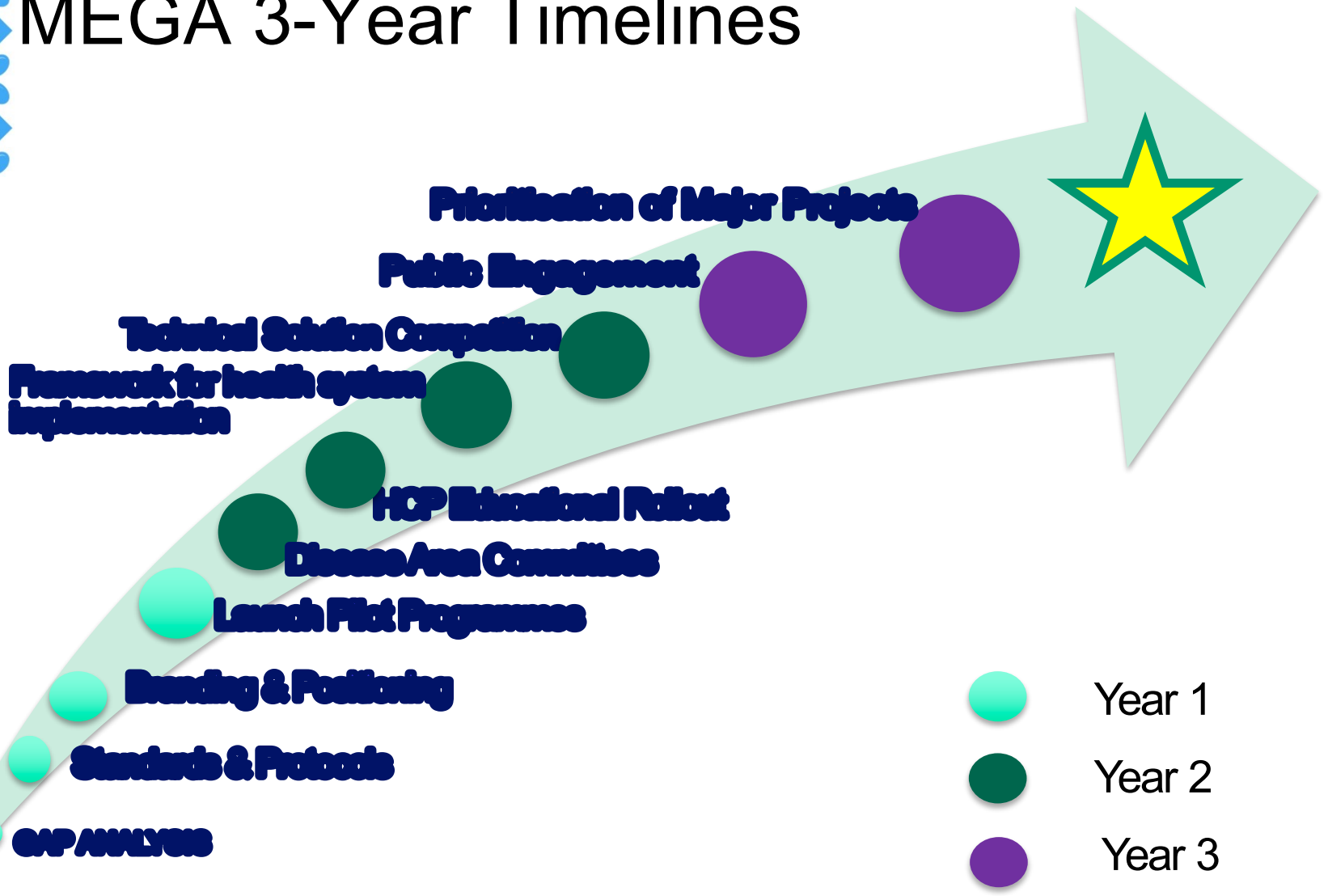
MEGA 3-Year Timelines



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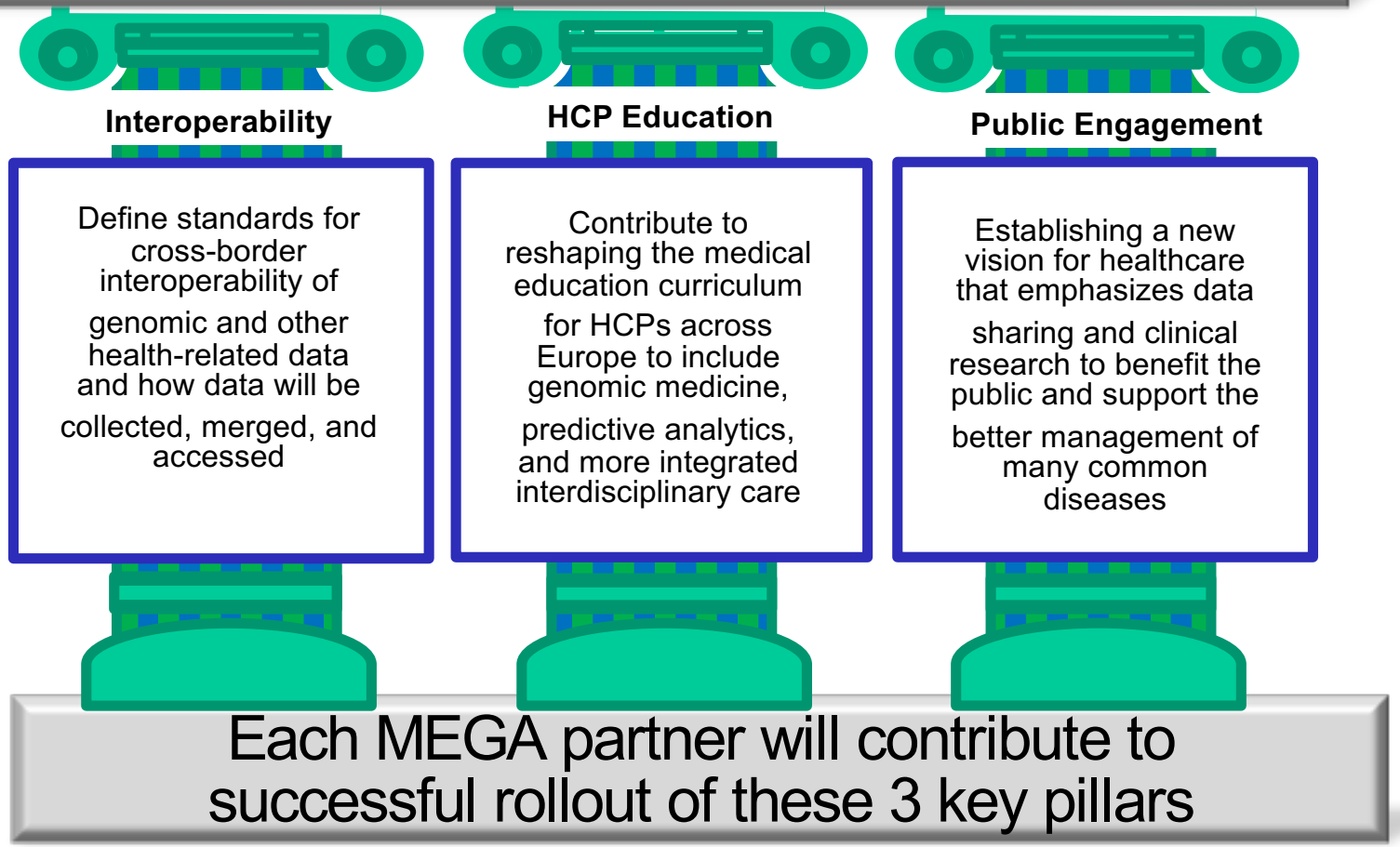
Million European Genomes Alliance



Each MEGA partner will contribute to successful rollout of these 3 key pillars

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Clarify the gap that needs to be closed by country and in relation to cross-country collaborative efforts by aligning the various stakeholders to facilitate delivery including relevant academic/research groups; commercial partners (large and small companies); clinical experts; patient groups; payers; and regulators.

To do this a series of Expert Committees will be established:

- **Committee 1. Clinical and Scientific**
- **Committee 2. Ethical Legal and Social Implications**
- **Committee 3: Education, Engagement and Communications**
- **Committee 4: Data and Informatics**
- **Committee 5: Sequencing**

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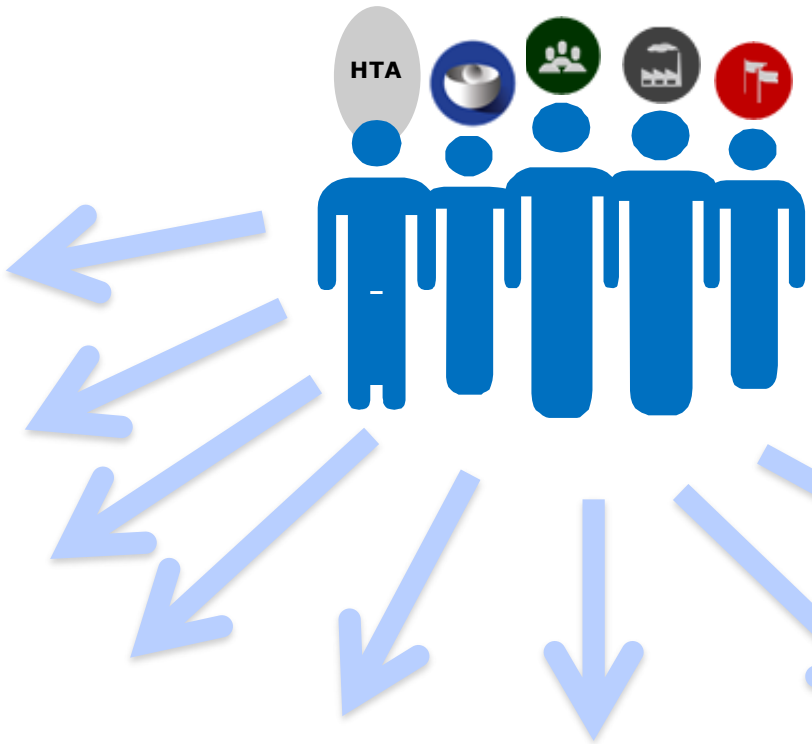
Benefits for Stakeholders



- Reducing cost of development
- Enabling innovation
- Optimise indications
- Safe, accelerated access to medicines

- New outcome measures
- Effectiveness data
- Improved EMA and HTA decision making

- Optimising use of medicines through ongoing monitoring
- Ability to define the impact of regulatory/HTA decisions
- Determining safety and efficacy in high risk groups
- Faster identification and assessment of safety issues
- Patient stratification for benefit and risk

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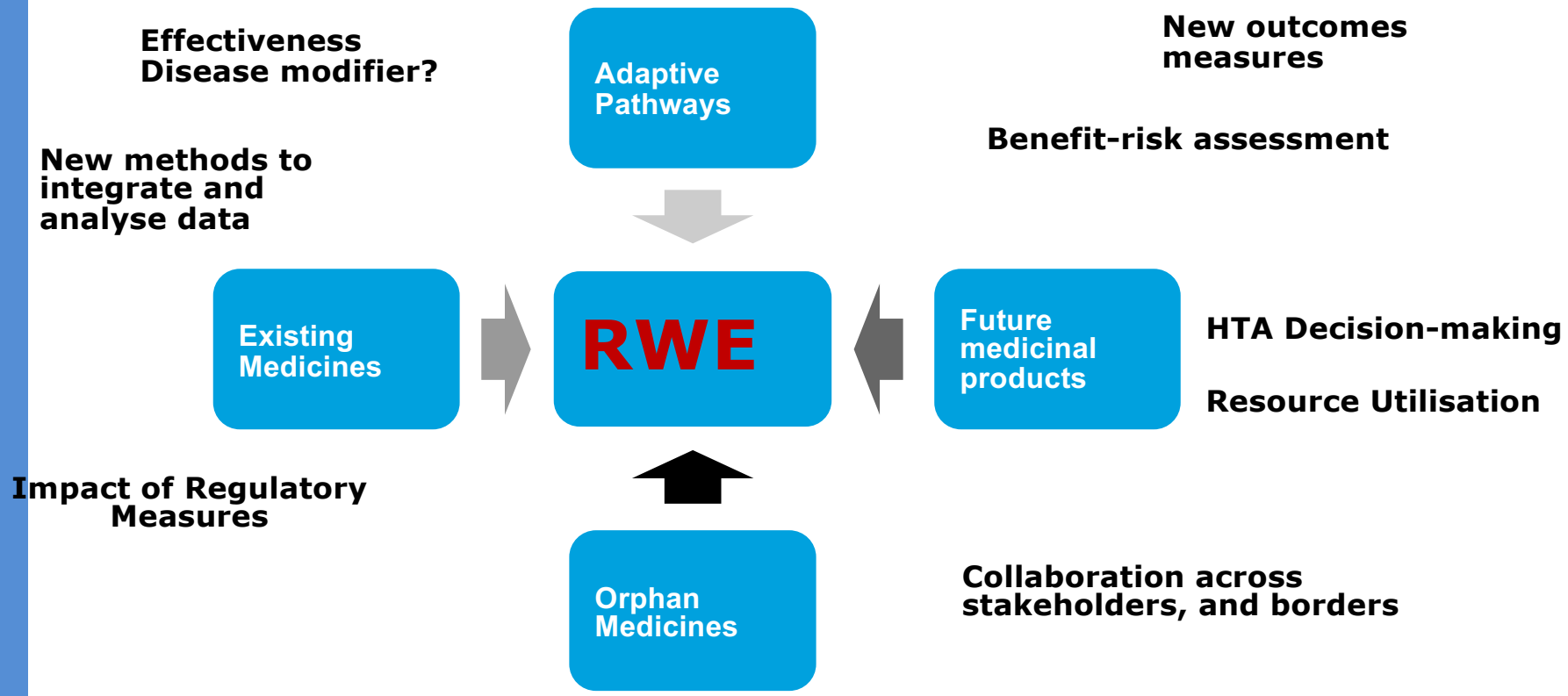
What is the Need?

RWE *throughout* Medicines Decision-making



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The Council Presidency can only achieve progress if its steps are based on the unity of the EU and its member states,"

Beate Hartinger-Klein, *Austrian Minister for Labour, Social Affairs, Health and Consumer Protection told the Committee on the Environment, Public Health and Food Safety, July 11 2018*

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Thank you!

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For more information about EAPM, please email:
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