European Alliance for Personalised Medicine

Barriers to Access in Personalised Medicine

EAPM briefing for the new legislative term, 2014

SIEPs

About EAPM

The European Alliance for Personalised Medicine (EAPM) brings together European healthcare experts and patient advocates involved with chronic diseases. The aim of the alliance is to improve patient care by accelerating the development, delivery and uptake of personalised medicine (PM) and diagnostics, through consensus building.

In response to the need for a wider understanding of the priorities arising from growing discussion on PM, EAPM was launched in March 2012 to foster a more integrated approach to the topic among lay and professional stakeholders. The alliance works on the development of case studies, education, training and communication material to deliver practical policy recommendations designed to exploit PM's full potential.

EAPM members provide extensive scientific, clinical, caring and training expertise in personalised medicine and diagnostics, across various stakeholder groups, from patients to academia, health professionals and industry. Relevant directorates of the European Commission have observer status, as £does the European Medicines Agency. By bringing together all of these stakeholders, EAPM's aim is to help forge constructive links between the EU institutions and society.

The EAPM Forum brings members together every 2-3 months to review activities and direct political strategy. Working groups develop positions on key topics and make proposals and recommendations to the Forum. The secretariat manages day-to-day operations, prepares Forum meetings, and co-ordinates the working groups.

Introduction

David Byrne, former European Health Commissioner and EAPM Co-Chair`

Personalised medicine is an exciting, innovative way to improve citizens' quality of life and produce better patient outcomes using the best science available. Yet, despite its many tangible advantages, the take-up in Europe has been relatively slow. This is not because personalised medicine doesn't work – it does, and very well - but it is because the components and interactions involved in bring individualised medicine and treatments to Europe's citizens are complex.

But bring it we must, as a healthier Europe will mean citizens spending less and less time in hospitals undergoing expensive treatment regimes, often at a direct cost to the taxpayer, and it will also mean that patients will be more able to continue working, thus generating wealth rather that whittling it away. By the same token, a shift towards preventative medicine will reduce costs still further.

Meanwhile, a focus on research into new medicines and cutting-edge treatments will also create jobs – whether they be in research itself, education, design and manufacture of in vitro diagnostic products or within the pharmaceutical industry.

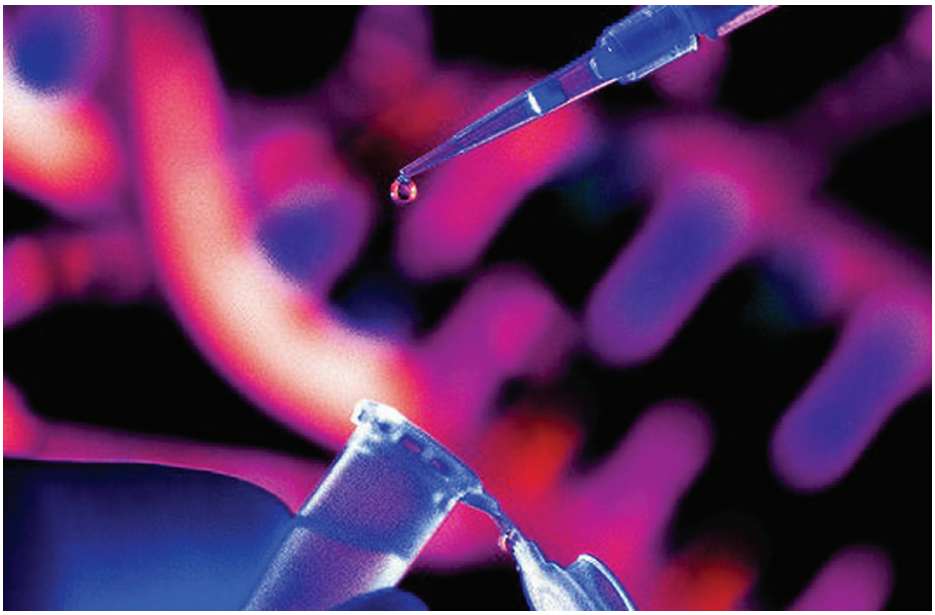
Such a focus will clearly benefit society and, if Europe is in the vanguard of developing new ways of keeping citizens healthy, it will inevitably attract investment from other continents.

However, there is an urgent requirement in many areas for reworking and rethinking in order to bring the practice of medicine up to speed in a fast-changing world.

EAPM has begun this process and has already achieved considerable successes. Not only has it brought together stakeholders that include patients' groups, clinicians, researchers, industry representatives, academics, policymakers and more, but the Alliance has also managed to have personalised medicine (PM) recognised and included in recent and upcoming legislation. This has happened in the areas of clinical trials, research and in the recent European Commission's Communication on the Data Driven Economy.

But much remains to be done to lower the barriers currently blocking the ability of PM to give the right treatment to the right patient at the right time, thus ensuring equal access to the best treatments available for all of the EU's 500 million citizens across 28 Member States.

Through multi-stakeholder consultation, EAPM has identified the key barriers as listed on the following pages.



Awareness

The awareness level of PM – its potential and its current application - needs to be raised, specifically among doctors who either do not know what it is, do not know enough about it or simply do not know whether it is available for their patients.

Training

Up-to-the-minute and continuous training for healthcare workers is urgently required. Any new knowledge needs to be clearly transmitted to the patient as this will greatly assist in bringing about their empowerment.

Patients

Europe's patients must be more involved in every aspect of their own health - from clinical trial remodelling to legislation to all other issues that affect them.

Collaboration

Stakeholders across all disciplines must learn to leave their 'silos' in order to interact. Cooperation and cross-

disciplinary collaboration needs to improve markedly.

Big Data

Biomarkers based on genetic data lead to ethical issues concerning the leaking of data. Legislation should take into account the specifics of PM and create an infra-structure in which genetic information is available in a regulated context. National and EU policies currently focus on data protection and privacy, while it is more desirable to focus on empowerment of the patients and ensuring access to their own data. Assuming that satisfactory ethical and workable legislation is put in place so that patients are comfortable with sharing their personal information, it then has to be collected, disseminated and, crucially, understood. There is a huge education gap when it comes to PM, even among some doctors, nurses and patients

IT and interoperability

The healthcare industry is perceived as slow to produce IT solutions and there are interoperability concerns in some areas that need to be dealt with. For example, interoperability between biobanks is hampered in the EU since different national frameworks exist, increasing the complexity of merging datasets. The national differences in legislation and regulations not only cause interoperability issues, but also have an impact at a financial level, in terms of reimbursement evaluation.

Verification

A PM-centric Europe needs to focus more on the verification of biomarkers at a high standard and with the consensus of relevant stakeholders. A lack of consistent evidence currently exists, which results in clinical uncertainty and presents a need for more targeted research.

Translation

A successful and efficacious chain that connects PM-research to PM-care and PM-treatment will require changes in healthcare infrastructure, clinical trials are run and organised, and how current diagnostic models are approached.

Research

The research approaches used in basic science do not meet the expectations of evidence needed to implement PM. This necessitates an improvement in the knowledge transfer between basic research and clinical research through the development of enforceable best practice guidelines. Moreover, as research into treatments takes a great deal of time - largely because treatments are often combined therapies, negotiations

between therapies. Negotiations between companies could be facilitated.

Standards

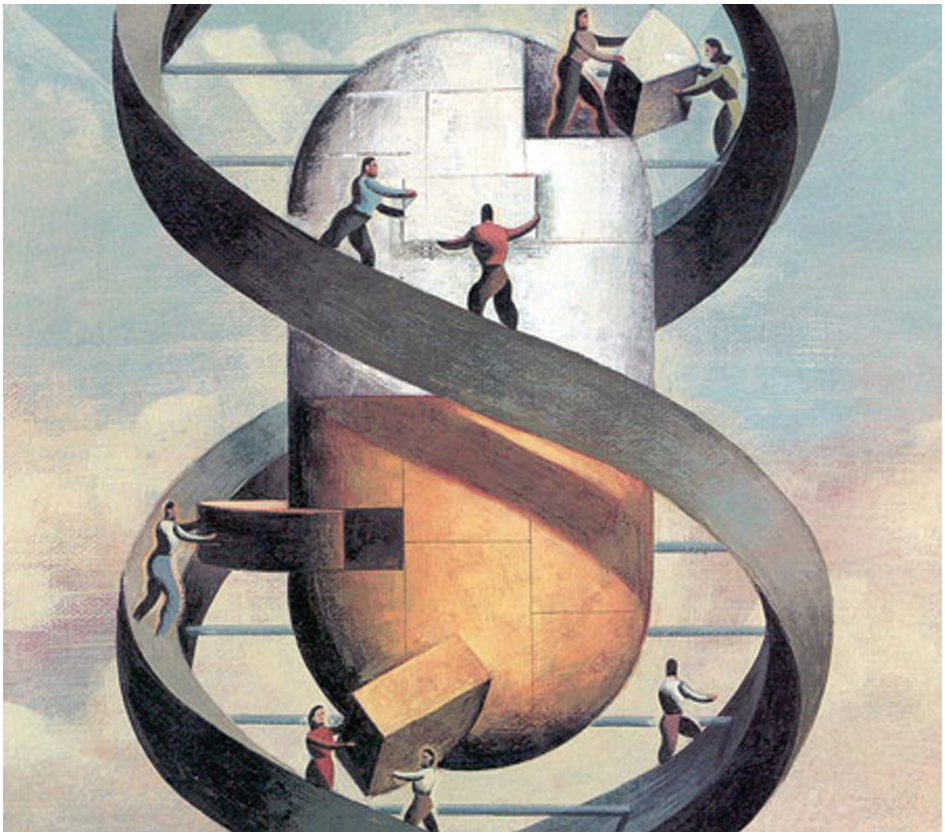
There is a need for stakeholder-agreed standards on everything from validating biomarkers to definitions of 'informed consent' to the use of IVDs. Best-practice guidelines must be formulated and enforced.

Regulation

PM needs to be regulated centrally to ensure its safe and effective use, because a lack of consensus in guidelines on interpretation and use currently exists. Regulations need to be robust and consistent while being flexible enough to adapt to a fast-moving field. At the moment regulations are not up-to-speed with the developments in science and industry. This results in outdated and inappropriate regulation. As an example, regulatory processes in respect of biomarkers need to be adapted.

Rewards versus viability

A new rewards and reimbursement model for technologies needs to be put in place, and for their part technologies must have proof of viability on a fiscal level. Whether the promise of PM can be realised depends, in part, on the existence of consistent evidence, a lack of which results in clinical uncertainty and a knock-on effect of scepticism, especially among payers. Conversely, confidence will grow



with every successful validation, intervention and effective treatment.

Reimbursement

There is a need to create an up-to-date and fit-for-purpose reimbursement model which encourages and rewards investment in good research, which lies at the heart of the future of EU healthcare. Without adequately funded and targeted research, the goal of true personalised medicine will be unattainable.

The above barriers can and must be successfully tackled if we are to create a healthier and, thus, wealthier Europe for this generation and those that will follow.

The EAPM STEPs campaign

STEPs stands for Specialised Treatment for Europe's Patients and EAPM has identified five STEPs towards a healthier Europe. This has the goal of securing patients' quality of life through Personalised Medicine

The Alliance calls on decision-makers to commit to the following essential STEPs for 2014-2019:

- STEP 1: Ensuring a regulatory environment which allows early patient access to novel and efficacious personalised medicine (PM)
- STEP 2: Increasing research and development for PM, while recognising its value
- STEP 3: Improving the education and training of healthcare professionals
- STEP 4: Supporting new approaches to reimbursement and HTA assessment, 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 across Europe.

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