European Alliance for Personalised Medicine

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2016 and all that: Time to look ahead...

Greetings, and welcome to EAPM's final newsletter of 2016. There's still plenty going on in the run-up to the holiday period, which we hope you will all enjoy when it comes around. And what a landmark year it has been…

With two recent polls causing seismic shifts in both Britain (Brexit) and the US (the election of Donald Trump as 45th president) it looks as though we're in for a chaotic period. Things are certainly going to change in the sphere of healthcare in America, with Trump pledging on various occasions that he will "repeal and replace the disastrous Obamacare" (known properly as the Affordable Care Act or ACA) saying it is "crazy", "doesn't work" and "doesn't make sense".

He also called it too expensive and wants more input from the private sector.

After the election, Trump seemed to soften his stance, but not by much. He said: "Either Obamacare will be amended, or repealed and replaced. I told [President Obama] I will look at his suggestions and out of respect, I will do that."

The president-elect went on to say that: "The administration recognises that the problems with the US healthcare system did not begin with - and will not end with the repeal of - the ACA."

We shall see…

Meanwhile, on this side of the Atlantic, despite all this new science, innovative and better ICT capacities and the ability to gather, store and disseminate Big Data, we are not making the most of it when it comes to giving the right treatment to the right patient at the right time.

A key reason for this is that much legislation is way behind the times and, until it gets up to speed, it will continue to constrict innovation.

We need a 'changing of the guard' in regulatory and legislative terms, as the old guard have concerns, worries and, it has to be said, prejudices that lead to caution in the face of radical advancement.

Many of our 'knowledge leaders' with influence in dozens of fields are simply behind the times and still taking us down the bumpy slow roads when the 'real' world has shifted to high-speed, overtaking lanes on technological superhighways.

It is very clear that emerging and fast-developing technologies (such as energy efficient building and genome sequencing) have facilitated human ingenuity and added to vital knowledge dissemination (often to the great benefit of medical research, for example).

EAPM believes that Europe cannot afford to kill off the positive knock-on effects of innovation. And if such innovation is disruptive and leads to those 'paradigm shifts', then all the better.

It's time for regulation in Europe to start keeping up.

In the pipeline

- **6 December: Big Data Working Group (WG)**
- **7 December: General Meeting of WGs**
- **17 January: Regulatory WG**
- **24 January: Education WG**
- **26 January (& 21 February): Big Data WG**
- **30 Jan-2 February: Engaging with MEPs**
- **7 February: The Challenge of Precision Oncology & Drug Development Processes**
- **14 February: Orphan Regulation - Catching Up in the PM Era**

Big Data Working Group

This Working Group will meet on 6 December to talk about the MEGA project and general data matters.

We are all making more-and-more use of data on a daily basis, and this is equally true when it comes to medical research.

The efficient gathering, storage and sharing of 'Big Data', while raising issues of data protection, ethical usage, technical requirements and more, will help us to investigate questions across a large number of diseases in different populations.

This would ultimately help in understanding clinical-care results and potentials in an individual.

Using Big Data, researchers theoretically have the ability to access millions of genetic markers and accelerate science towards a better understanding of diseases.

These data and the huge advances in genomics form just a part of the personalised medicine revolution, albeit a vital part. And these important components are tied together: all that information is of little use unless it can be shared and exploited by researchers for the benefit of patients today and those that will follow tomorrow.

As a response to US **President Barack Obama**'s Precision Medicine Initiative (PMI), is the proposed launch of EAPM's MEGA project. MEGA stands for the Million European Genomes Alliance and will act as a link between genome sequencing efforts across Europe, with the goal of compiling a huge database for medical research.

MEGA comes on the back of America's One Million Genome project, which Obama launched with an initial $215-million, and will work with a pool of people, both healthy and sick, men and women, old and young, to expand knowledge of how genetic variants affect health and disease.

EAPM's executive director, **Denis Horgan**, said: "The gene genie is already out of the bottle and, regardless of who is running things politically on either side of the Atlantic, the stopper cannot be put back in. Now is the time for Europe to react to Obama's PMI with its own ambitious initiative."

MEGA has already gained support from Intel, with **Mario Romao**, its digital health senior policy manager for Europe, quoted as saying that MEGA is in line with Intel's own 'All in One Day' concept.

With this Intel aims to bring about an environment in which clinical teams can diagnose cancer or other genetically-based diseases, plus conduct secondary analyses within 24 hours. The aim is to achieve this by 2020.

EAPM's plan for a Committee on Health Data Access and Accessibility, meanwhile, has the aim of creating a formal expert committee under the Commission's governance, with representation from all relevant stakeholder groups, including Member State bodies.

General Meeting of Working Groups

One day later, on 7 December, the Alliance will meet to see and hear presentations from the different Working Groups in order to get an overview of past and future work.

EAPM's Working Groups consist of Big Data, Education and Training of Healthcare Professionals and Stakeholders, Access/ Value, Research Roadmap for Personalised Medicine, MAPPs and a Regulatory Affairs Taskforce.

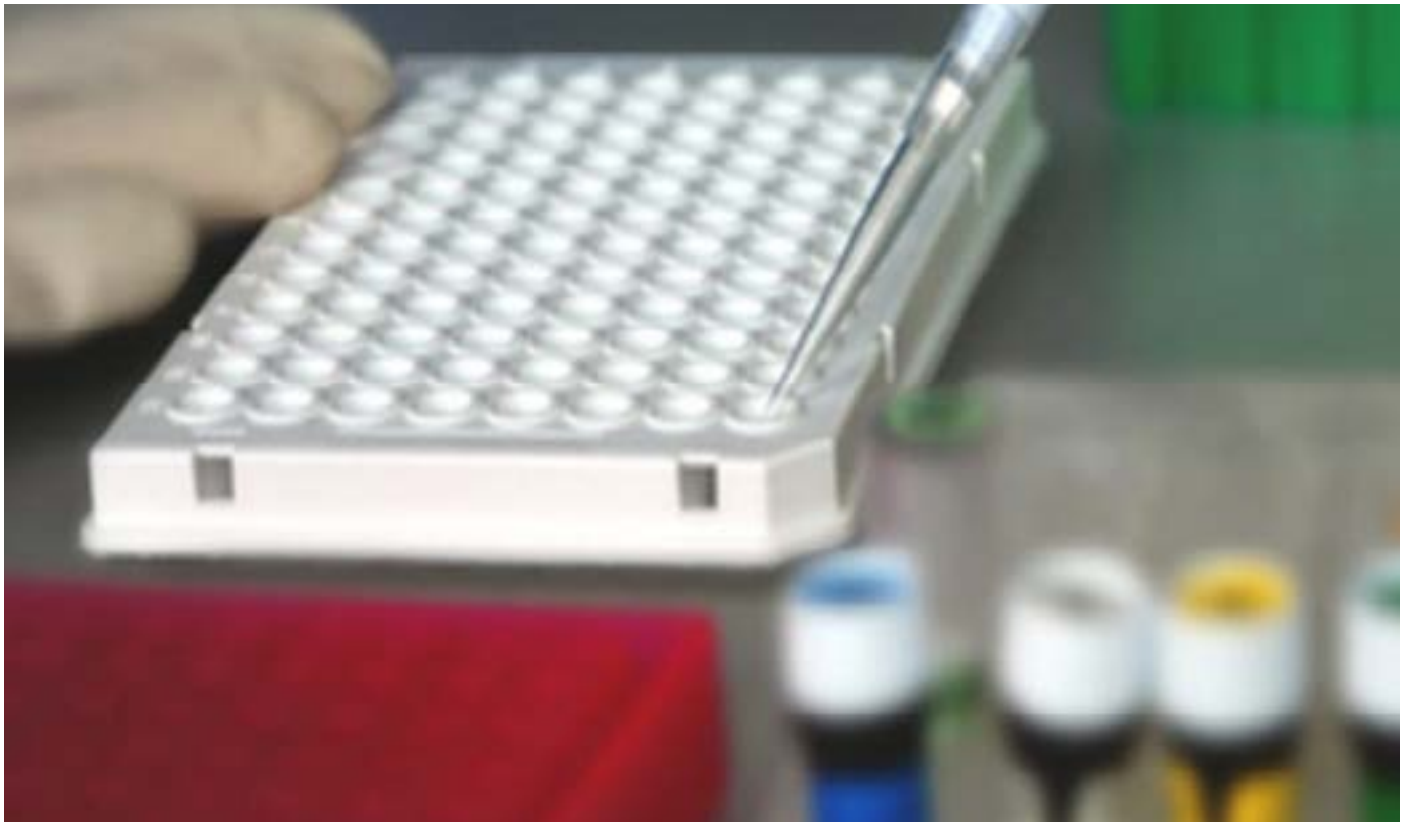
These groups bring together stakeholders in neutral forums in order to discuss the issues related to their specific area (for example, patients' unmet needs, medical perspectives, research, industry and more).

The goal is to understand any differences, overcome any misunderstandings and to find common ground, leading to a prioritisation of issues.

Activities include parliamentary engagement at the EU and Member State level, research engagement, the writing of policy papers, as well as meetings with institutional representatives.

Achievements within this framework have included key changes to three pieces of legislation (Data Protection, Clinical Trials and In Vitro Diagnostics), supporting research in terms of Horizon 2020 and IMI II legislative proposals, plus putting personalised medicine on the political map through the Luxembourg Presidency's Council Conclusions of a year ago.

During this meeting, presentations will be given for Belfast Congress in late 2017, the Lung Cancer Screening Conference in March and EAPM's second Summer School, to be held in Bucharest in July.



IVD Meeting

On 24 November, an Alliance meeting was held in Brussels on the topic of 'Navigating our way in the new regulatory terrain of In Vitro Diagnostics medical devices in Europe'.

The all-day gathering featured three sessions and covered the Role of EMA and interaction with notified bodies and national competent authorities, dialogue with healthcare stakeholders, and the impact of new regulation on in-house tests.

Speakers at the event included **Jesús Rueda**, Director of Regulatory Affairs at EDMA, **Vincent Houdry**, Legal Officer, DG for Internal Market, Industry, Entrepreneurship and SMEs, **Falk Ehmann**, Scientific Support & Projects at the European Medicines Agency, **Sue Spencer**, Head of Global Medcial Device Services, UL, and **Laura de Vries**, Dutch National Authority.

They were joined by **Stanimir Hasurdjiev**, Access Partnership, European Patient Forum, **Ian Banks**, president of the European Men's Health Forum, **Giovanni Codacci Pisanelli**, Assistant Professor of Medical Oncology at Rome University, **Mario Pazzagli**, Professor of Clinical Biochemistry, Department of Clinical and Experimental Biochemical Sciences at the University of Florence, and **Jonathan Truelove**, Senior Commercial Counsel EMEA, Genomic Health.

During the meeting EDMA's Rueda said that IVD proposals are going in the right direction but there is a need for international harmonisation - the approach taken in the EU is different from that taken outside. In the US a single authority handles everything, while there are some aspects of the EU system which are particular to us. This could create barriers.

Ian Banks, meanwhile, said that there is very variable access across EU and asked whether the regulatory process would facilitate access. He added that it would be a very sad day if an effect of the regulation were to be to slow down innovation.

The meeting included lively question and answer sessions and was introduced by Denis Horgan.

Access to Medicines

On 11 November, in the Brussels European Parliament, a discussion was held (during the ENVI Committee meeting) on the back of a September draft report on EU options for improving access to medicines.

The draft had been circulated by the EP's Committee on the Environment, Public Health and Food Safety, which has as its rapporteur **Soledad Cabezón Ruiz**.

At the time, EAPM said it was certainly welcomed, but added that the Alliance is of the opinion that, while the draft report addresses broad issues in respect of medicine pricing, transparency and more, it does not cover many other necessary areas, not least prevention as well as over- and under-treatment (a growing issue), the regulatory system that stakeholders have to work within, and other 'symptoms' affecting EU healthcare systems that need to be properly diagnosed and treated quickly.

In the end, there were more than 600 amendments. Here are just a selection:

Sirpa Pietikäinen added a Motion for a resolution Recital B a: "Whereas patients should have access to the healthcare and treatment options of their choice and preference, including to complementary and alternative therapies and medicines."

The same EAPM-supporting MEP added a Motion for a resolution Paragraph 12 a: "Welcomes the Commission's work on creating more indicators to measure the outcomes of health care operations; Calls for the inclusion of added therapeutic value of a drug as an indicator when deciding on the reimbursement of medicines, as it happens that often drugs and personalised medicines with a higher price tag bring savings for the health care system in the longer term due to their more rapid efficacy."

And **Andrey Kovatchev**, **Cristian-Silviu Buşoi**, **Kateřina Konečná**, **Biljana Borzan** and **Karin Kadenbach** added a Motion for a resolution Paragraph 13: "Believes that prices of medicines should be adequate to the specific economic situation of the country where they are marketed."

During the November meeting Cabezón presented the report, which was followed by further interventions from 18 MEPs.

Cabezón especially noted the need to find an appropriate balance between the sustainability of healthcare systems in Europe as well as to ensure access to medicines for citizens and the need to guarantee pharmaceutical innovation.

She added that these correspond to the right to access to care and intellectual property rights and highlighted the existing gaps in priorities set for research and the unmet needs in some therapeutic areas.

The rapporteur also mentioned the lack of supply and shortages of medicines in some countries, which hamper access to drugs for patients. Member States have a key role in guaranteeing access to medicines as they are responsible for price setting, reimbursement and allocating funds to research.

The European Parliament has the duty to give the political incentives to improve the situation, Cabezón stressed.

MEPs who took the floor after the presentation mainly welcomed the report, but some expressed concern over the wording used in some parts of the report, which they felt was bashing industry.

Karl-Heinz Florenz (EPP, shadow rapporteur) especially noted that industry has led to many improvements and new treatments, referring to the development of orphan drugs.

Meanwhile, MEPs from the S&D group expressed their support to the content of the text, some of them noting that the "dark picture" of the situation showcased in the report unfortunately depicts the reality.

Karin Kadenbach underlined that it was the role of legislators to develop the regulatory framework that would prevent unaffordable prices.

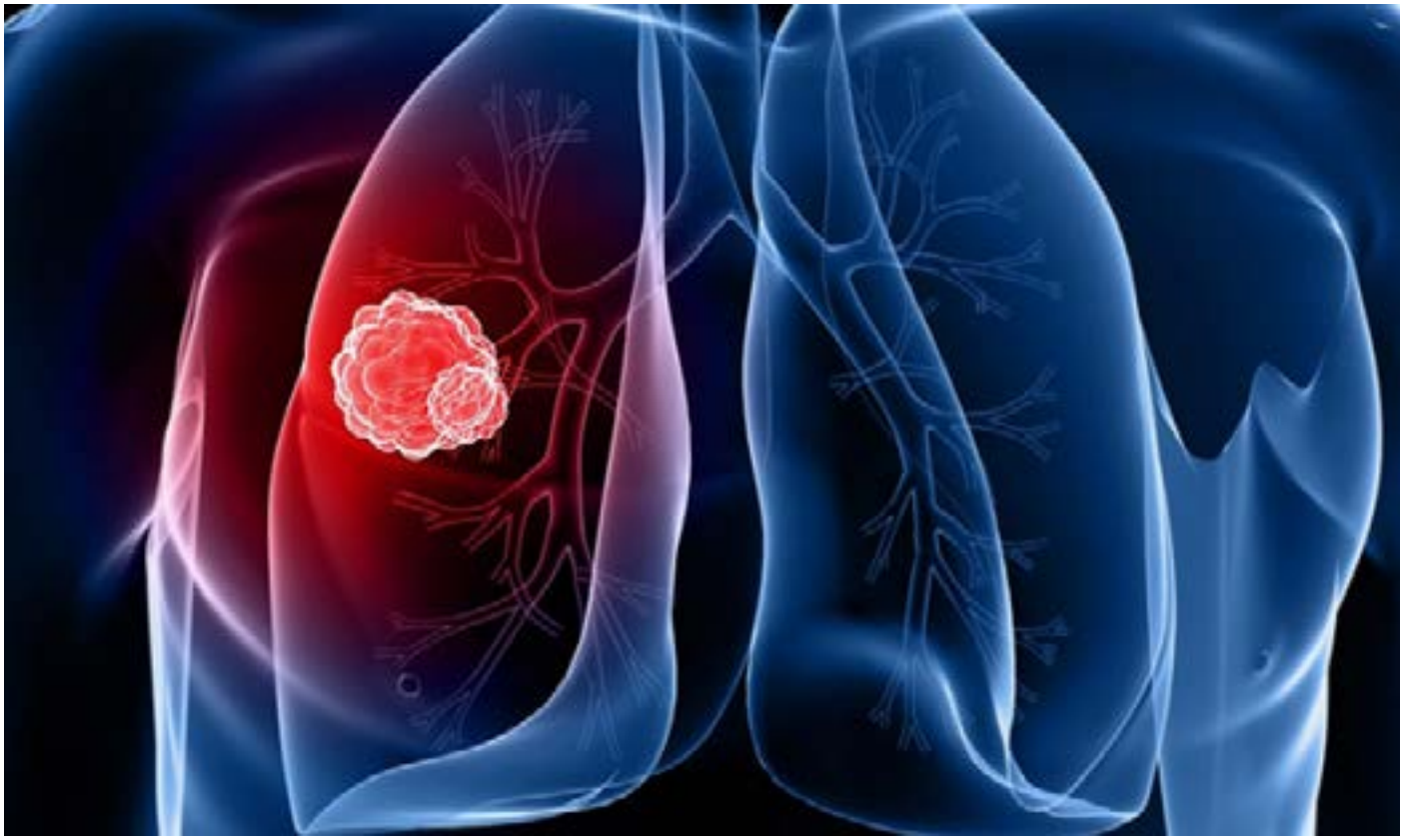
Meanwhile, European Commission representative **Dominik Schnichels**, Head of Unit B4 "medical products: quality, safety and innovation" in DG SANTE, provided an overview of the Commission's activity in the field.

On intellectual property rights, he noted that patents provide a very good incentive for research and in many cases ensure a return on investment. In line with the June Council Conclusions, the Commission will analyse the impact of incentives.

As regards Health Technology Assessment, he referred to the Impact Assessment on the issue recently launched to consider possible initiatives on HTA, but stressed that any action would need to respect the distribution of competences between the Commission and Member States, meaning that pricing and reimbursement will remain national competences.

He added that the Commission also supports Member States in cooperation and exchange of information on HTA.

Concluding the discussions, MEP Cabezón explained that the report did not call for uniformed prices across Europe but aimed to ensure that Member States have tools to establish prices and determine reimbursement system in the interest of patients.



Looking ahead

This year has been a very busy one for EAPM and, of course, 2017 will be a lively year too. So first let's look ahead to the Alliance's fifth annual conference to be held in March under the auspices of the Maltese Presidency of the European Union.

Entitled '**Innovation and Screening in Lung Cancer - The Future**', the conference, to be held at the historic Bibliothèque Solvay in Brussels on 28 March, will build on solid foundations and aim to raise awareness among policymakers about the needs of modern-day patients and how personalised medicine has the potential to change healthcare for the better.

High-level speakers and attendees will come from a wide range of stakeholder groups including patients, healthcare professionals, academics, industry representatives, politicians and legislators, the media and more.

The conference will be held across one full day, and the aim is to see real and concrete recommendations emerge.

Modern medicine is advancing swiftly and there are many areas trying to play catch up. With the giant leaps in gene sequencing, imaging, data availability and more, a genuine improvement in cooperation is required across all disciplines and all geographical areas.

Much can be achieved with consensus-based guidelines to ensure that all stakeholders are aware of acceptable standards and are effectively all 'singing from the same hymn sheet'.

There is clearly a need to:

- Raise awareness of the need for agreed guidelines over lung-cancer screening

- Improve the knowledge of policymakers and world health agencies so that effective lung-cancer screening guidelines and policies can be formulated on the international stage

- Work across national borders to ensure cooperation and collaboration in respect of much-needed guidelines in the fast-developing field of personalised medicine

- Advance parallel work done by professional groups, patient groups, healthcare funders, pharmaceutical companies and academic institutions to a new level

Figures show that lung cancer causes almost 1.4 million deaths each year worldwide, representing almost one-fifth of all cancer deaths. Within the EU, meanwhile, lung cancer is also the biggest killer of all cancers, responsible for almost 270,000 annual deaths (some 21%).

It is at the very least surprising that the biggest cancer killer of all does not have a solid set of screening guidelines across Europe.

Obviously, doctors need to quickly identify high quality, trustworthy clinical practice guidelines, in order to improve decision making for the benefit of their patients.

Undoubtedly, tobacco smoking is the major risk factor for lung cancer, although passive smoking, and a family history of lung, head and neck cancer are, among other factors, also important.

Around one billion people on the planet are regular smokers. And, as noted, lung cancer is one of the biggest killers. We all now know that there is a direct connection in many cases. Non-smokers do get lung cancer, but the risks if you are a smoker are significantly higher

As mentioned elsewhere in this newsletter, the Alliance's Working Groups cover many health areas, and it has now turned some of its attention to the need for more guidelines in

screening for lung cancer, and there is a need for agreement and coordination across the European Union's 28 Member States.

Given that health is in the main a Member State competence, when it comes to governance it is clear that national structures need to be in place for screening. These would benefit from EU-wide guidelines, political commitment, and a structure that provides for evidence-based decision making (the latter in a fully transparent manner).

In these testing financial times, with expenditure on health-care rising, it is vital that the benefits of screening are weighed against potential harms (radiation etc) and cost effectiveness. Patients need to be made aware of potential negatives of screening in their case (as well as the benefits) and thus be able to make a choice.

Co-decision making is a core principal of patient groups and other proponents of personalised medicine.

Summer Scool 2017, Bucharest

July 2017 will see EAPM host its second, week-long summer school for young healthcare professionals, or HCPs.

It will be co-chaired by **Richard Ablin**, Professor of Pathology at the University of Arizona College, who first observed the antigen PSA as long ago as 1970.

PSA levels are now used as a test for prostate cancer, from which his father had died.

Despite this, Dr Ablin has since famously said that the US effectively wastes billions each year giving preventive prostate cancer screening tests that produce, in the majority of cases, false positives.

Ablin maintains that doctors and patients should be cautious when using PSA as a marker for preventive screening.

With such a high-profile figure as co-chair, alongside former European Commissioner for Health **David Byrne** (who is also co-chair of EAPM), the second summer school is well set to build on the success of the first event, held in July 2016 in Portugal.

Next year, the concept will be taken east to Bucharest, (above) in Romania, with the school taking place from 3-7 July.

The choice of Bucharest dovetails with EAPM's SMART Outreach project. SMART stands for Smaller Member states And Regions Together, and EAPM has been expanding this by taking its message directly to EU countries.

Once again, the school will be entitled 'TEACH', which stands for Training and Education for Advanced Clinicians and HCPs, and the goal is to bring young, front-line professionals up-to-speed with fast-moving developments in the field.

Aimed at age-range 28-40, TEACH holds to the thesis that, if personalised medicine is to be in line with the EU and Member State principle of universal and equal access to high-quality healthcare, then clearly it must be made available to many more citizens than is currently the case.

The faculty has been chosen from medical academic, clinical and research specialties, patient organisations and communication experts.

One session, focused on implementing science and technology for the best care, will discuss current and future guidelines, the role of regulators, better tools in pathology and, not surprisingly, the pros and cons of screening.

And Big Data will indeed be big at the summer school. The huge topic of the gathering, storage and sharing of medical data - and the need for a common framework with which to share it - will be covered in depth, including ethics and concerns from legislators and the public alike.

Furthermore, finding the balance, or middle ground, between new science/technology and medical tradition is not easy, so another tutorial session will take a look at various aspects of this.

A key goal in the personalised medicine era is to improve communication between front-line HCPs and their patients. The latter should have an equal role in any decisions made about

their treatment, and this requires them to be able to input vital information, such as lifestyle and work circumstances, as well as to be properly informed from the other side.

Therefore, time at the summer school will be spent on health literacy, clinical decisions involving patients and improving the communication between core actors of health decision making.

In the news

As ever, EAPM has been busy in the media. Click the links below to read our more recent articles.

This is the modern world – Time for regulation to move with the times

Frustrated patients deserve better health-care strategies

Democracy – Would you vote for it?

Trump: Do or not do after legitimate debate?

Twelve steps to help cure post-election ills

Trump: Total rewrite of US medical policy?

Value in modern-day medicine. Who decides?

Battling disease can be even harder than you think

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About EAPM

The European Alliance for Personalised Medicine (EAPM) , launched in March 2012, brings together European healthcare experts and patient advocates involved with major chronic diseases. The aim is to improve patient care by accelerating the development, delivery and uptake of personalised medicine and diagnostics, through consensus.

As the European discussion on personalised medicine gathers pace. EAPM is a response to the need for wider understanding of priorities and a more integrated approach among distinct lay and professional stakeholders.

The mix of EAPM members provides extensive scientific, clinical, caring and training expertise in personalised medicine and diagnostics, across patient groups, academia, health professionals and industry. Relevant departments of the European Commission have observer status, as does the EMA. EAPM is funded by its members.

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