European Alliance for Personalised Medicine

EAPM Bulletin: Issue 14, May 2016 www.euapm.eu

A great conference, and the work continues...

Welcome to our May newsletter.

EAPM would like to thank everybody who attended our highly successful 'Taking Stock' conference (our fourth annual event) in Brussels last month, as well as those of you who followed the gathering on social media. We are already planning the fifth annual conference for 2017.

Opening the event, EAPM's co-chair, Helmut Brand, told the audience: "Despite the tragedy of the Brussels bombings, we are delighted to see many friends here. Nearly 80% of those who registered are here. This is an important figure because it shows it will not let us stop our lifestyle and our work to improve modern society and its values, going forward and not backwards.

He added: "And there is a second issue - in recent times we have seen the rise in euro-scepticism and nationalism. But research, innovation and healthcare are good ambassadors for European cooperation. Personalised medicine can be the 'best practice' example for this."

A full conference report will be available in due course.

In the pipeline

- **1-2 June: PerMed conference on personalised medicine, Brussels**
- **June (dates to be confirmed): SMART Outreach meetings in Spain and Portugal**
- **June (dates to be confirmed): Meeting on orphan medicinal products and special edition on Access for All**
- **4-8 July: Summer school for HCPs, Cascais, Portugal**

Roundtables on research and innovation

Post-conference, EAPM co-ordinated a two-day series of roundtables in Brussels, with key Member State, Commission and wide stakeholder input, on five key challenges associated with research and personalised medicine.

The event took place over Monday and Tuesday, April 11-12 and was geared towards feeding into a Commission/ICPerMed conference on personalised medicine to be held on 1-2 June, in the Belgian capital.

EAPM will organise a follow-up meeting later in the year to further these important topics.

The workshops followed on from the PerMed 'Strategic Research and Innovation Agenda' (SRIA) on Personalised Medicine, launched at a press conference last June.

The Commission, Member States and EAPM, alongside other stakeholders, are striving to develop a roadmap for research that aims to embed personalised medicine approaches into European health systems and acknowledges the need to identify "exemplars" which can be tested as models for effective translation of research into clinical benefit, as well as showing "added value".

Europe not only needs world-class research, it requires a regulatory framework, and an economic model, that will allow new drugs not only to be developed but also to be made available to patients who need them, wherever they need them, across Europe.

Citizens need to benefit from better co-ordination of research with all stakeholders involved, including a cross-section of legislators in the European Union.

Europe is still at the very early stages of translating research results into actual products. Research is key, as is a focus on innovation in health care.

Attendees at the workshops were made up of researchers, academics and high-level experts on many aspects of personalised medicine. The five crucial areas discussed were:

Developing awareness and empowerment

Through personalised medicine, the role of HCPs and patients will evolve. Patients and healthcare professionals need to develop the required awareness and a first step is to deliver best available evidence supporting the clinical and personal utility as well as the economic value of new approaches to health systems.

Casa de Santa Maria: one of the TEACh Summer School venues

Translating basic to clinical research and beyond

In order to achieve its anticipated impact on the health and well-being of citizens, translation of discoveries and communication across the continuum of research are required. This starts with the integration of all the 'omics' data to generate and implement meaningful interventions and diagnosis.

Shaping sustainable healthcare

Personalised medicine will rely on healthcare systems that are able to adapt to these approaches in a timely and socially acceptable manner, while enabling the participation of all stakeholders to increase effectiveness.

As mentioned, training for health professionals is required as is the promotion of engagement and close collaboration between all stakeholders, including patients.

Integrating Big Data and ICT solutions

The development of personalised medicine will rely heavily on integrated Big Data analytics and ICT solutions to generate and integrate the required knowledge and infrastructure for new approaches. Technologies for data capture as well as the management and development of high-quality databases will be as instrumental as strategies to make sense of data for known and future purposes.

Bringing Innovation to the market

Innovative solutions represent a higher uncertainty regarding going to market. There is a need to develop new risk-based approaches for their evaluation in a context that encourages systematic early dialogue with all stakeholders providing guidance for companies.

It is clear that patients today are more aware of the clinical improvements that can be achieved through the use of personalised-medicine tools such as biomarker tests. Patients need empowerment, which means good access to information, and an ability to participate fully in discussions about the management of their disease.

Shared decision-making should become a reality and is part of empowerment, but it is also about shared knowledge and, not least, education. Which brings us to…

Summer School for HCPs

Coming up soon is EAPM's first Summer School for young healthcare professionals, taking place in July 2016.

Entitled 'TEACH' (Training and Education for Advanced Clinicians and HCPs), the school will be held at various cultural venues (see below) in Cascais, near Lisbon, Portugal, and will run from 3-7 July. Four museums have been chosen as the venues for the summer school sessions.

Personalised medicine is now at the centre of most, if not all, aspects of patient care. It is not limited to rare diseases or cancer, but spans all medical specialties. This exciting new way of treating patients is, however, based on specific concepts or biological pathways in a field which is continuously moving.

This means that all healthcare professionals (HCPs) in close contact with patients or their families need to possess a solid knowledge of the current aspects of personalised medicine and its latest breakthroughs, in order to better understand patients' concerns.

The Alliance is firmly of the belief that health-care professionals cannot be expected to adapt to new ways of approaching patients and coping with new technology unless they are suitably trained.

These HCPs are being asked to move beyond traditional reactive medicine towards proactive healthcare management, employing screening, early treatment, and prevention, and to classify and treat diseases in a new way, interpreting information from across sources that blur the traditional boundaries of individual specialties.

Professionals will need to be confident of the science behind targeted therapies, including greater understanding of the immune system and molecular medicine, and knowledge of the

mechanisms of action and interaction of targeted therapies, as well as common adverse events.

To this end, the Alliance will run its summer school aimed at bringing 80 young professionals and patients (aged 28-40) up to speed with doctor-patient communication skills.

The summer school also aims to support the endeavours of EAPM to set up a Continuous Educational Programme on personalised medicine.

The training will consist of plenary sessions followed by time spent in small groups focussing on how to communicate in respect of several defined topics over the course of four days.

Tutors have been chosen from medical academic, clinical and research specialties, patient organisations and communication specialists.

The school has its own dedicated section on the EAPM website, so see more and register, here

European Commission/PerMed meeting

EAPM will be involved in the previously mentioned European Commission/ICPerMed conference on personalised medicine which will be held on 1-2 June.

As we all know, many of our most common medicines are not effective in treating large numbers of the patients they are supposed to help and more than six percent of acute hospital admissions are caused by serious adverse reactions to medicines. Moreover healthcare costs across the EU are rising as the population ages and chronic diseases become more prevalent.

Personalised medicine addresses these challenges, with tailor-made prevention and treatment strategies for individuals or groups of individuals. As a result, patients receive the specific therapies that work best for them, and no money is wasted on trial and error treatments. Personalised medicine is a fast-growing market and Europe's healthcare industry has the potential to build on its leading position, providing economic growth and jobs.

The Commission is currently exploring ways of improving collaboration in funding of personalised medicine research in Europe. Hence, in parallel to several ongoing international research initiatives, the EU executive has started discussions with funding organisations in Member States about setting up a joint collaboration in this field. One of the recent EU-funded project with particular policy relevance to personalised medicine is PerMed.

The PerMed project gathered a wide range of partners, with the main aim of producing a Strategic Research and Innovation Agenda (SRIA) for personalised medicine. The SRIA contains a number of research recommendations grouped into five challenges ranging from creating awareness about personalised medicine to shaping sustainable healthcare.

The recommendations are aimed at public and private organisations active in the area of health and health research.

SMART Outreach

The Alliance's June 2015 conference introduced the 'SMART' concept, which stands for Smaller Member States and Regions Together, and EAPM has been expanding this by taking its message directly to EU countries.

Successful outreach events have already been held in Poland, Austria, Bulgaria and Italy and more are planned this year at venues in Spain and Portugal, coming up in June.

Although Brussels-based – which helps to better engage with the European Commission, EU permanent representations and the European Parliament in the 'Capital of Europe' – EAPM believes it is time to place its feet firmly on the ground in more EU countries, in order to expand its work with the multi-stakeholder groups, and nations, that form its membership.

Roundtable session at the 4th annual EAPM conference. Photo from www.simonpugh.com

Our Outreach programme has already been a great success and, rest assured, the Alliance is building on that.

Special issue on Access for All

A special issue of *Public Health Genomics* in June will address the topic of "Access for All".

In December 2015, during the Luxembourg EU Presidency, the Council of issued its Conclusions on Personalised Medicine for Patients, highlighting how "the development of personalised medicine may offer new opportunities for the treatment of patients in the European Union … allowing healthcare providers to offer better-targeted treatment, avoid medical errors and reduce adverse reactions to medicinal products."

Readers will know that personalised medicine certainly has the potential to improve outcomes for European citizens, but its undoubted promise must be balanced against a number of highly relevant challenges that may limit its positive impact on 21st century medicine.

Issues such as increasing costs, inequitable access across European countries and regions and the need for a PM-relevant ethicical, regulatory and reimbursement environment are currently undermining PM integration at European and national levels.

Given the need to address these important issues, EAPM assembled a multi-stakeholder panel to identify and precisely define the critical barriers that limit personalised medicine uptake, while also developing solutions that will enhance patient access to this new form of treatment across Europe.

The output from the discussions is captured through a series of articles in this special issue. The articles complement the activities of the EAPM's Working Group on Access, which is surveying the European landscape and developing policy recommendations in this complex domain. The topics covered are as follows:

- Access for all: A personalised approach
- Shooting for the Moon or Flying too near to the Sun? Crossing the Value Rubicon in Precision Cancer Care
- The value of companion diagnostics: overcoming access barriers to transform personalised healthcare into an affordable reality in Europe
- Enabling equal access to molecular diagnostics: what are the policy and health technology assessment implications?
- A Conceptual Framework of Mapping Healthcare Access across EU Countries: The Patient Acces Initiative
- Accelerating the Development and Validation of New Value-based diagnostics by Leveraging Biobanks
- Gene Tailored Treatments For Brain Disorders: Challenges and opportunities
- Personalised medicine and scarce resources - A discussion of ethical chances and challenges from the perspective of the capability approach
- Early patient access to medicines: Health Technology Assessment Bodies need to catch up with new Marketing Authorisation methods

Meeting on orphan medicinal products

EAPM took the opportunity to comment during the public consultation of the Commission's regulation overseeing orphan medical products. This was in February.

Images from the 4th annual EAPM conference (and European Parliament dinner) in April. Photos from www.simonpugh.com

A meeting will be held to further discuss the issues and the Alliance has stated that, in the fifteen years since the introduction of the Regulation, there has been significant progress and changes in drug development in particular for small patient populations.

But it is important to recall that the spirit of the original Regulation is to stimulate the development of new orphan drugs providing a medical benefit that is meaningful to patients suffering from rare conditions.

EAPM appreciates the intention to further clarify the definition of "significant benefit", particularly with regard to the way sponsors need to demonstrate 'significant benefit' over authorised medicines. Nevertheless, this should be done with caution. The Commission must be mindful that in the development of treatments for small patient populations, conclusive data may not be available at the early stages of the drug development process.

It is important that the revised text recognises the validity of methods other than direct comparative head-to-head studies such as clinical cohort studies.

EAPM noted that not all drug development can be considered breakthrough and important progress can often only be achieved by incremental treatment improvements as seen in the case of childhood leukemia, where small incremental improvement has led to a 90% success rates over time.

Clinically relevant advantage, such as efficacy, can be measured with different endpoints. For instance, in cancer, progression free survival and overall survival are frequently used.

The debate continues, and EAPM will be at the heart of it, as ever.

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.

Contact: Denis Horgan
EAPM Executive Director
Avenue de l'Armee/Legerlaan 10, 1040 Brussels
Tel: + 32 4725 35 104
Website: www.euapm.eu