European Alliance for Personalised Medicine

EAPM Bulletin: Issue 12, March 2016 www.euapm.eu

Alliance closes in on Spring conference

Welcome to EAPM's March newsletter. The Alliance has a busy spring and summer ahead including its annual conference, a series of SMART Outreach events and a new Summer School for young healthcare professionals (HCPs).

4th annual conference

The 2016 conference is coming up fast and will be held once again in Brussels at the historic Bibliothèque Solvay on 5-6 April.

As ever, it aims 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.

The theme will be: "Taking Stock: Where we are now and the necessary next steps."

Given that personalised medicine has never been more in the public eye than it is now, especially in the wake of US President Barack Obama's initiative on precision medicine, the conference aims to reflect this.

The Americans are moving quickly and have come to some obvious conclusions including that health equals wealth and that investment in research and innovation, alongside laws and rules that are fit-for-purpose and reflect the swiftly changing world of medicine, are vital.

Europe needs to grasp these points at every level – not just the vision of an EU that creates a competitive environment that attracts investment, but also for the benefit of the millions of potential patients spread across 28 Member States.

At Bibliothèque Solvay, 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.

As in previous editions, the conference will be held across one-and-a-half days, and the aim is to see real and concrete recommendations emerge.

There is now only one month left in which to register, and you can do so, here

TEACH Summer School for HCPs

July will see the first in an annual series of ground-breaking TEACH summer schools on the subject of personalised medicine.

In the pipeline

- **1-2 March: SMART Outreach, Warsaw**
- **7 March: SMART Outreach, Milan**
- **4-5 April: Pre-conference genomics event, in partnership with Illumina,, Brussels**
- **5-6 April: 4th Annual Conference, Brussels**
- **11-12 April: Research WG roundtables, Brussels**
- **4-8 July: Summer school for HCPs, Cascais, Portugal**

TEACH stands for Training and Education for Advanced Clinicians and HCPs (healthcare professionals) and is an Alliance initiative.

The goal is to bring young HCPs up-to-date with developments in this exciting new field.

Aimed at healthcare professionals aged 28-40, TEACH will cover topics such as monoclonal antibodies, inhibitory drugs and putting the patient at the centre of his or her own care - all within the context of personalised medicine.

The gathering will be held from 4-7 July in Cascais, Portugal, and, over the course of the four-day school, a 20-strong faculty of experts will oversee plenaries, group discussions and interactive role play sessions involving the +/- 80 HCPs enrolled on the course.

Among other aspects, the summer school will provide a forum for presenting new data from clinical trials and basic research and sharing ideas for innovation.

It will allow its attendees to enhance their knowledge of evidence-based approaches on diagnosis and treatment, let them access the latest results on clinical and translational research, and bring them up-to-speed on emerging innovative techniques, diagnostic tools and more.

Bibliothèque Solvay, which will once again host EAPM's annual conference

Christine Chomienne, past president of the European Hematology Association and a driving force behind the scheme, believes that, in the changing world of health care in Europe, including exciting new developments in personalised medicine, the education of health care professionals is under-emphasised.

She said: "The true potential of all of this fantastic new science, built around genetic profiling and individual DNA, will never be fully realised unless front-line clinicians have the knowledge and understanding to exploit it."

HCP/patient relationships will be key. In the run-up to the recent Luxembourg Presidency Council Conclusions on Personalised Medicine, the Grand Duchy's health minister, Lydia Mutsch, said: "The exciting field of personalised medicine is, and should be, all about the patients. It offers the opportunity for them to be seen not merely as passive recipients of care but as participants, partners and even guides in their own healthcare."

She added that one important goal should be "giving clinicians better tools to treat and inform their patients and allow HCPs a better understanding of their patients' needs".

Professor Gordon McVie, a cancer specialist and EAPM secretary, took up the theme: "The issue of education of HCPs is a major one. It is clear that a great degree of up-skilling is already required and, to keep pace with the science, this must be ongoing," he said.

"Europe must ensure that no patient is denied a suitable, virtually tailor-made treatment due to a lack of knowledge or understanding on behalf of the HCP treating and diagnosing him or her."

Meanwhile, EAPM treasurer and leading urologist Didier Jacqmin said: "There is certainly a need for ongoing education among HCPs in this exciting new era of healthcare in order to make the most of the recent explosion in technology.

"Given the advances in personalised medicine in recent years there is now a need to reform how healthcare is delivered to the technology-aware patient. One way to achieve the goal is through the education and training of healthcare professionals. The summer school is just the beginning."

On education generally, EAPM has called for action at EU level, saying: "By 2020, the EU should support the development of a Europe-wide education and training of health care professionals' curriculum for the personalised medicine era. The EU should subsequently facilitate the development of an Education and Training Strategy for HCP in Personalised Medicine."

Research roundtables

EAPM's Working Group on Research is organising a series of meetings on 11-12 April on how to drive the health research and innovation agenda for years to come, with a view to improving healthcare.

The workshops will showcase the current state-of-the-art in the area and explore future research and innovation challenges. Also under discussion will be how to ensure new and better collaborations among health research funding and policy making organisations - the International Consortium for Personalised Medicine (IC PerMed).

The workshop series is being developed with Member States' collaboration on the five thematic sessions below:

- Developing Awareness & Empowerment
- Integrating Big Data & ICT Solutions
- Translating Basic to Clinical Research & Beyond
- Bringing Innovation to the Market
- Shaping Sustainable Healthcare

The workshops will draw participants from a variety of stakeholder angles and organisations including patient and civil society representatives, researchers, industry representatives, funders, policymakers, healthcare payers, and health practitioners.

Outreach events

EAPM's latest SMART Outreach meetings are taking shape and aim to set up an Alliance presence at national levels.

The Alliance's 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.

All outreach events follow on from EAPM's original STEPs campaign – Specialised Treatment for Europe's Patients – which calls for the EU to commit to:

- STEP 1: Ensuring a regulatory environment that allows early patient access to novel and efficacious personalised medicine
- STEP 2: Increasing R&D for personalised medicine, while also recognising its value
- STEP 3: Improving the education and training of health care professionals
- STEP 4: Supporting new approaches to reimbursement and HTA, required for patient access to personalised medicine
- STEP 5: Increasing awareness and understanding of personalised medicine.

Successful outreach events have already been held in Poland, Austria and Bulgaria with two more coming up – Poland, for a second time (1-2 March in Warsaw), and Italy (7 March in Milan).

Click here for the agenda for Italy and here for the agenda for Poland.

These agendas reflect the barriers to fully integrating personalised medicine into EU healthcare systems, not least of which is patient access.

Speakers in Milan will include Gianluca Vago, Rector of University of Milan, Beatrice Lorenzin, Italian Ministry of Health, Mario Melazzini, President of Italian Medicine Agency, and David Byrne, former European Commission for Health and EAPM Co-Chair.

Speakers in Warsaw include Prof. Zbigniew Gaciong, Chair, Polish Association for Personalised Medicine, Prof. Jacek Fijuth, Chair of the Polish Oncology Association and a member of Polish Alliance for Personalised Medicine, and Jacek Graliński, Public Affairs Director, AstraZeneca Poland.

A further event will have an 'all Ireland' focus and take place in Belfast on 26 April, followed by EAPM on the ground in Spain, Portugal, Greece, Germany and France in May and June.

Orphan Regulation consultation

EAPM has recently welcomed the opportunity to comment on the Commission Consultation concerning the Application of Regulation (EC) No 141/2000 on orphan medicinal products.

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.

The Alliance believes 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.

It gave the following responses to various consultation points;

Clarification of the definition of "significant benefit"

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.

The CHMP 'Guidance on Clinical Trial in Small Populations' recognises that ' in conditions with small and very small populations, less conventional and/or less commonly seen methodological approaches may be acceptable if they help to improve the interpretability of the study results'.

EAPM welcomes the reference in the Notice to adaptive clinical trials. However, stakeholders are advocating greater flexibility in the drug development model, moving towards a more flexible system with smaller, smarter clinical trials that rely on the much more effective upfront research performed with data and patient material and take into account patient-reported outcomes, even potentially as measureable endpoints.

Unfortunately, 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.

In certain conditions (e.g. Chronic Myeloid Leukemia, CML), it has been proved that treatments are well tolerated by the patients if they are taken during the course of his/her lifetime. They allow patients to have almost normal life expectancy compared to the average citizens. EAPM strongly recommends, specifically for chronic conditions, that it is emphasised that even in case of status quo in terms of comparison to the existing treatments, life transforming treatments can be clearly considered as having significant benefit over maintenance or palliative treatments.

Encouraging the development of orphan medicinal products for communicable diseases (e.g. Ebola)

EAPM agrees with the current proposal.

Simplifying the procedure for the reassessment of orphan criteria when two authorisation application procedures are pending in parallel for two orphan medicinal products

EAPM welcomes the wish of the Commission to be flexible in case of procedures assessed in parallel. However the proposal for one month is restrictive and it would be practical to extend the timeframe to allow for the data generation for significant benefit comparison.

Introducing the reassessment of the orphan criteria for a new subset of the condition when a sponsor extends the use of its product after marketing authorisation

The intention to verify if the new therapeutic indications are of significant benefit when compared to existing treatments is understandable. There is a need to clarify if the failure to demonstrate significant benefit for a new sub-set would have impact on the original indication.

For example, as the term of 're-assessment' may imply that the previously demonstrated significant benefit for the initial indication may be re-questioned, which would be detrimental for the development of orphan medicines and ultimately for the advancement of precision medicine.

This is also not in line with the concept of "adaptive pathways", for which sponsors are encouraged to target a first approval in the population with the highest unmet medical need and to further pursue additional subsets.

Academic researchers explore new orphan indications and they may sometimes gather the proof of significant benefit. However, academic networks have frequent difficulties in convincing private partners to enlarge the treatment indication for a particular drug or therapy.

EAPM recommends putting in place a specific type of advice by the European Medicines Association whereby academic researchers may ask guidance on whether evidence gathered or to be gathered would demonstrate the required significant benefit, or if additional research is needed, so that an appropriate public-private partnership has the best chance of success. To read more, click here

Genomics for health

The European Alliance for Personalised Medicine (EAPM) and Illumina, a world leader in genomics, will hold an inaugural 'Genomics for Health' meeting at Bibliothèque Solvay, Brussels, from 4-5 April, immediately prior to its own event on 5-6.

This prestigious event will bring policy makers together with those leading the implementation of the young and exciting science of genomics into healthcare.

Where genomics in health stands today - and its incredible potential through Next Generation Sequencing and more - will be presented, backed up by discussions on key challenges to progression, differing perspectives and the development of a high-level plan for advancement in this crucial and swiftly moving area.

Topics to be covered during the event will include improving treatment selection in cancer, maximising success of diagnosis in rare diseases, and what (regulatory) framework is best to support innovation in genomics.

Also, during the gathering, the 'Healthcare Genomics 2030' initiative is to be launched as a campaign driven by the Brussels-based EAPM and Illumina.

The initiative's aim is to engage and inform European Union and Member State policy makers in order to shape the

Milan - location for an upcoming SMART Outreach event

landscape for the successful implementation of genomics and related technologies in the healthcare arena.

The two-day 'Genomics for Health' meeting will bring these policy makers together with thought leaders from healthcare, academia, industry, and patient organisations under the banner 'Determining a path for optimal integration of genomics into healthcare across Europe'.

Genomics is the foundation that enables the vast potential of personalised medicine to be realised, much of it preventative.

With rising healthcare costs and individual health systems being increasingly challenged, genomics has the potential to impact the health of all of us and provide diagnostic, economic and efficiency benefits, ensuring that patients receive the right information and the right treatment at the right time.

This will ease the burden on healthcare systems and lead to a healthier and, thus, wealthier, Europe. The potential for bettering the health of the EU's 500 million citizens is huge.

To register for the event, please click here

In the news

As ever, EAPM has had several articles published in the media recently. Simply click on the titles below.

Apple gets to the core of the data privacy debate

Personalised medicine advocates reach out to Poland and Italy

Rare diseases – looking better in genes

Almost there – gene technology in the health arena

Leaving EU is not best plan for a healthy Britain

World Cancer Day highlights need for cost-effective approaches to new medicines

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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