European Alliance for Personalised Medicine

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Commission outlines changes to HTA

Welcome to February's newsletter. The new year is behind us and 2018 is now well underway. There are many challenges to be faced by Europe in general, not least in respect of the Brexit negotiations, and many to be faced by those of us involved with personalised medicine, including the all-important patients.

Access is still sub-optimal, HTA is facing a shake up (more of that to follow), cooperation across borders still needs to be boosted, medical data needs to arrive at its required destinations and our healthcare professionals need to be brought up to speed with advances in science, to name but a few.

Of course, the Alliance has input into all areas outlined above and has a busy year ahead. Your hard work is greatly appreciated and your commitment highly valued.

HTA in the spotlight

The European Commission has a new proposal on plans for future cooperation among EU countries on determining the added value of new therapies.

This outlines mandatory use of joint clinical assessments reports, after a three-year transition period, and was issued publicly in final form on 31 January.

It could go a long way to allowing necessary medical bodies to catch up with a scientific arena that is moving forward quickly.

By the end of the transition period, "all medicinal products falling within the scope and granted marketing authorisation in a given year will be assessed". This also covers selected medical devices.

What the Commission is looking for in this case includes "patient-relevant health outcomes," and a "degree of certainty" of those effects.

This is welcome news. Major advances in healthcare are just around the corner, with a wealth of new possibilities promised for European patients and society through the increased adoption of personalised approaches to medicine.

EAPM believes that, if the potential of personalised medicine is to be realised, changes are necessary in the way medicines are developed and regulated.

The bottom-line is that systems need to catch up with science and we are in ongoing discussions with MEPs on this topic.

In the pipeline for 2018

- **22 February: Members Meeting: Discussion on Incentives & HTA, Brussels**
- **27-28 March: 'Personalised medicine and the Big Data challenge', Sixth Annual EU Presidency conference, Brussels**
- **10 April: The Digital Day 2**
- **23 April: 'Lung Cancer Screening - Moving forward towards guidelines', Sofia**
- **19-22 June: Third TEACH Summer School for healthcare professionals, Warsaw**
- **26-28 November: Second Annual EAPM Congress, Milan**

Under the Commission's proposal, HTA bodies in Member States would be required to use the clinical assessment and "no repetition" of it in their overall processes.

Aside from that, the HTA bodies may issue non-clinical determinations, for example in the areas of ethics, economics, or other factors that may influence value. These will be put together with EU-wide analysis before issuing a final assessment.

At the moment, while most drugs in the EU are centrally authorised by the soon-to-be Amsterdam-based European Medicines Agency, critical assessments and related decisions about the value of medicines come under Member State c ompetence.

A key aim of the new Commission proposal will be to reduce duplicated work across the bloc.

It is an urgent matter for HTA bodies to make effective re-use of HTA information from one Member State to another, to reduce duplication of work and provide consistency across territories which, as noted, appears to be a key aim of the Commission proposal.

On 22 February we will hold an EAPM members meeting for a discussion on incentives and HTA.

Just to recap, EAPM and its members and stakeholders have consistently called for:

- Better understanding among HTA bodies of the specificities of the -omics technologies
- Adaptation of assessment methodologies to take account of new ways of developing evidence
- Integration of the concepts of overall economic value and equity into HTA flexible CT designs
- Wider input from stakeholders into HTA bodies' methodologies

* Greater cross-border sharing of expertise among HTA bodies

- An EU-wide HTA standard method to support developments in personalised medicine
- Closer alignment of the assessment of therapies and companion diagnostics
- Advice and support, particularly to smaller firms, on how to prepare for HTA requirements
- Early engagement in terms of dialogue and advice with the HTA agencies

6th Annual Presidency Conference

Our sixth annual conference will, like the first five editions and the inaugural Congress last year in Belfast, pull together leading experts in the arena drawn from patient groups, payers, healthcare professionals plus industry, science, academic and research representatives.

We are delighted to announce that European Commissioners Vytenis Andriukaitis, (Health and Food Safety) and Andrus Ansip (Digital Single Market) will also be joining us.

The theme this year will be '*Personalised Medicine and the Big Data Challenge*' and the conference will once again be held in the historic Bibliothèque Solvay in Brussels on 27-28 March.

The draft agenda is available to view, here, and registration will open next week.

Key topics at the conference will include EAPM's MEGA project, which stands for 'Million European Genomes Alliance' and aims to gather one million genomes from across Europe for research purposes.

The Alliance is, as ever, continuing its dialogue with MEPs on this issue.

The availability of genetic data from a large number of individuals increases the ability to investigate questions across many rare and common diseases and in different populations, and also provides more information for understanding the results for clinical care in a patient.

MEGA aims to form a coalition of the willing Member States to work together and reach the million genome figure.

A coordinated, pan-European MEGA project would garner crucial genetic information that could have an immeasurable benefit when it comes to the health of current and future citizens across the EU.

EAPM is now in discussions with Member States to make this dream a reality.

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6th Annual Conference

BRUSSELS

Plans for the sixth annual Presidency conference of the Brussels-based European Alliance for Personalised Medicine are already in place for 27-28 March 2018.

The 'Personalised Medicine and the Big Data Challenge' event will be held under the Bulgarian Presidency of the EU, which runs from 1 January to 30 June.

Taking place close to the Brussels seat of the European Parliament, the conference will feature plenary sessions in the afternoon of Day One, followed by a dinner in the parliament that evening and a day-long event on Day Two.

The conference will revisit the prestigious Bibliothèque Solvay in Parc Leopold.

The effect on healthcare of Big Data, across many disciplines, will certainly mean that clinical researchers and other healthcare stakeholders and professionals will need to develop new expertise and a different approach. Ongoing training will be vital, but there are many other issues to be discussed.

Given the European Parliament elections, which will be not much more than a year away at the time of the conference, a key goal will be to raise awareness of personalised medicine in respect of current MEPs who will be standing again, and potential new Members.

We would be delighted to have you join us in Brussels.

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More data to treat more diseases

A further conference session will raise and discuss the topic of '*Realising the Vision of Personalised Healthcare through Big Data*' while another will cover the broad picture of profiling, genomics and personalised healthcare.

On a wider personalised medicine basis, Europe needs synergies to be realised at Member State level, a topic to be discussed at length while specific disease areas, such as haematology and Big Data, will come under the Alliance microscope.

Medical research, clinical trials and more are generating unprecedented amounts of Big Data that is moving treatments forward in many disease areas. However, rare diseases present their own challenges, and in this sense the need for cross-border, pan-European collaboration is greater than anywhere.

Using the latest WHO classification, rough incidence rates for European haematological diseases showed that differences in diagnostic and registration criteria in The EU Member States are an important cause of incidence variation, and that certainly data on haematological issues across the bloc needs to improve.

Those who work in the field of haematology believe that a single point of access for data would go a long way to building a comprehensive knowledge base for rare haematological diseases.

It would allow researchers an easily accessible, quick European overview of disease prevalence and affected populations, as well as vital socio-demographic data. Member State collaboration and European Commission support is vital in this context.

Big Data can also be put to excellent use by providing the evidence base for treatments, not least in neurology, which will be discussed at the conference, alongside public health genomics and diabetes.

More related to MEGA

The European Commission has prepared a briefing note which outlines the next steps in any 'coalition of the willing'.

This will include the proposed signing of a declaration on 10 April. What fantastic news!

MEGA's ultimate goal is to compile a database of a million genomes for clinical research purposes, via Member States joining forces, as well as for stimulating the life sciences economy, and improving patient care across the EU.

A key element of any support will be a willingness by ministers and MEPs to sign the declaration.

As the Commission puts it, among the goals is to link "existing and future genomic databanks (on a voluntary basis) in order to reach a cohort of one million sequenced genomes accessible in the EU by 2022".

This joint commitment will make it possible to bring together fragmented infrastructure and expertise supporting a shared and tangible goal.

The project is in line with the Digital Single Market mid-term review priorities, and supports the realisation of the aims expressed in the Luxembourg EU Presidency's Council Conclusions on Personalised Medicine.

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2nd Annual Congress, MILAN

The European Alliance for Personalised Medicine (EAPM) is already planning a second major, personalised medicine Congress in Milan on November 26–28, 2018.

Similarly to the inaugural 2017 Congress in Belfast, this will be a pan-European, multi-disciplinary event specific to the fast-moving field of personalised medicine and will take place from 26–28 November 2018

EAPM and its stakeholders believe that Europe needs to build better healthcare systems for its current 500 million citizens, and the generations to come.

A key aim of the Congress is to allow cross-fertilisation between the different disease and policy areas, allowing delegates to gain a greater depth of knowledge into barriers in the field of personalised medicine.

It is also geared towards offering up valuable evidence and stakeholder opinion on which policy makers can base their decision making on how better to integrate personalised medicine into the EU's healthcare services.

The event will provide the biggest 'space' to date to allow for such a meeting of minds and expertise, and EAPM is building a one-stop-shop for top-level discussion and the formulation of real action plans. So be sure to come.

Once again, the Congress will bring together the different streams (including scientists, industry, regulators, patients and more) in order to allow decision makers to understand changes that are required, now and down the line.

The Congress will pull together leading experts in the arena of personalised medicine drawn from patient groups, payers, healthcare professionals plus industry, science, academic and research representatives.

For more information, please contact the EAPM Office:
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On top of this, at the end of 2017, the Council adopted conclusions on 'Health in the digital society – making progress in data-driven innovation in the field of health' inviting the Member States and the Commission to: "Build on the European Cloud Initiative, the EuroHPC and the European Open Science Cloud."

EAPM can confirm that several Member States have already indicated that they find this project of special interest to them - a 'mega' step forward.

Congress heads to Milan

The second annual EAPM-run Congress will complement the upcoming sixth annual Presidency Conference in Brussels, and the first annual Congress (held in Belfast, November 2017).

The event will take place in **Milan from 26-28 November**, 2018, and we hope to see you there.

Meanwhile, the report from last year's Congress in Belfast is available online, here.

Third Summer School on the way

EAPM's third annual Summer Summer School will be held in partnership with the Polish Alliance for Personalised Medicine and will take place in **Warsaw from 19-22 June**. The agenda is almost finalised and will be available to view very soon.

Once again, the school will come under the banner '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, communication and research specialists. The opening of registration will be announced in due course.

Questions, questions...

EAPM will be soon launching a survey on incentives/rare diseases, in order to gauge the responses of different stakeholders on various issues.

This will include questions such as: 'To what extent do you think that the incentives introduced by the Orphan Drug Regulation have been effective in fostering research and development for rare diseases where no treatment or satisfactory treatment existed before?', and 'To what extent would you agree with the following statement: despite higher prices, rare disease therapies continue to represent a small fraction of pharmaceutical budget in my country.'

Your responses are extremely important, so please do look out for the survey.

Data, data, everywhere…

The General Data Protection Regulation (GDPR) will enter into force in May and EAPM is following this with considerable interest, as are our national affiliates. We expect engagement to become even more regular than it is already as the new era kicks in.

The Commission is already at work trying to ready Member States for the introduction of GDPR on 25 May .

Justice Commissioner Věra Jourová has been widely quoted as saying: "We need modern rules to respond to new risks, so we call on EU governments, authorities and businesses to use the remaining time efficiently and fulfil their roles in the preparations for the big day."

As it stands, a mere two Member States - Austria and Germany - have fully adapted national laws to take into account GDPR. We watch with considerable interest, and not a little concern…

In the news

EAPM will be producing a series of written pieces for the media and our discussion groups under the umbrella title of '*Just imagine…*'.

These will explore possible scenarios and ideals in the context of personalised medicine.

Meanwhile, below you can find links to recent articles.

Inequitable health care – a one-way ticket to undermine the EU

How Horizon 2020 is acting to advance personalised medicine

Age shall not wither them – facing the co-morbidity challenge

Saving lives through Big Data collection and usage

2018 – Let's make 'better health for all' a New Year's resolution

Keep it as simple as possible – and improve patient lives

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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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