European Alliance for Personalised Medicine

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A new year, and new challenges for all

Welcome to 2019 and our first newsletter of the year. All at EAPM wish you a happy and prosperous 12 months ahead and hope that you had an enjoyable and restful holiday period.

Of course, we all start every year knowing it's going to be a busy one. Arguably, however, 2019 will be even more so.

Not only do we have the issue of Brexit - in what ever shape it eventually takes - we also have European Parliament elections coming up in May followed by a new Commission towards the end of the year.

All of these are additional reasons why the Alliance, its members and partners will be stepping up activities to even higher levels at EU, Member State and also regional levels to get our key asks across.

EAPM has always employed the multi-stakeholder approach to ensure that all suggestions to various policymaking bodies at all levels come about through consensus, and this will continue.

The very specific topics for 2019 as mentioned above, as well as many others, featured heavily at our 2nd Annual Congress, held in Milan recently, and you can check out the daily newsletters from the event, plus the post-event report, below.

- Congress Newsletter, November 29
- Congress Newsletter, November 28
- Congress Newsletter, November 27
- Congress Newsletter, November 26
- Milan Congress Report

We also now have a new six-month rotating presidency of the EU, namely Romania, which will be taking on various challenges and wrap-ups, not least concerning health technology assessment (HTA), medical devices (more of both, below), and an over-arching philosophy of cohesion as a common European value.

Social cohesion is a biggie for Romania, which it says should should contribute to ensuring benefits, guarantees and equal chances for all EU citizens.

Citizens must be reassured concretely that will live better lives and have more access to the benefits in the EU rather than outside it, Romania says.

In the pipeline:

- **8-9 April: EAPM 7th annual presidency conference, Brussels**
- **19-22 June: 4th annual Summer School for HCPs, Leuven**
- **18-20 November: EAPM 3rd annual Congress, Brussels**

EAPM will be following and engaging on all of the above and more at policy level, as it has done successfully since its formation.

The thorny issue of HTA

Meanwhile, the upcoming Romanian presidency is keen to avoid the political battle over mandatory versus voluntary aspects of the Commission's plans for joint action on HTA.

Good luck with that, as a number of countries are against any mandatory element in this regard while a larger number, plus the European Parliament, think it is necessary.

A compromise seems a long, long way away and it may be left to the Finnish presidency, which takes over in July, to finish the job.

Romania's health attaché **Stefan Staicu** has said that his country wants to side-step tackling the relevant section in Article 8, moving on to Article 9 instead.

The latter is more about timelines.

Romania has set its sights on a technical compromise first, followed by a political one at the end of its presidency.

It remains to be seen how much progress Romania will make before the next Health Council meeting on June 14. Though last week Romania's Health Minister **Sorina Pintea** said the aim was to "reach a general approach at Council level".

The country's predecessor in the presidential chair, Austria, had aimed to secure at least a partial general approach by last month.

However, Austrian health attaché **Philipp Tillich** said: "As it turned out we had to adapt our objectives to the reality."

He described as "unfortunate" the fact that Austrian could ultimately only release a progress report, adding that there was "a very strong political resistance from a couple of Member States".

Tillich went on to advise Romania to "simply ignore" the heated political debate for the time being and backed the country dedicating itself to "those less tricky but also technically very important parts of the text".

EAPM has followed and participated in this on-going HTA debate since the Commission first floated the proposal early in 2018, and this will also continue going forward.

A look at what's been happening

Medical devices

Just before the Christmas period came an update (well, sort of) on the situation surrounding the implementation of medical device regulations, Single Market Commissioner **Elżbieta Bieńkowska** (pictured) had felt that some notified bodies tasked with assessing whether medical devices are safe and effective would be re-approved before the new year.

"There are still question marks," according to **Françoise Schlemmer**, head of the notified bodies' association Team-NB. Schlemmer added that things are going smoothly and probably will be "earlier than expected a few months ago", according to a report in *Politico*.

The original feeling was that fully authorising the notified bodies, necessary to approve medical devices, wouldn't happen until around the start of the Finnish presidency, which as mentioned above begins in July.

Drug pricing

The World Health Organisation (WHO) published a report in late December that picks holes in arguments from pharmaceutical companies in respect of their pricing of cancer drugs. Access campaigners will take note of the 171-page dossier that warns of what WHO calls "unintended consequences" in relation to orphan drugs.

This is because research and development incentives for rare cancer drugs often lead to, albeit unintentionally, companies pursuing an orphan indication initially before expanding to non-rare indications to, as WHO puts it, "gain faster market entry at high prices".

The report also adds that "cost-containment measures undertaken due to the high costs of cancer medicines, irrespective of population needs, have resulted in reduced, delayed and even cancelled treatment".

WHO's pronouncements also call for more transparency in respect of R&D costs and pharmaceutical prices, while expressing the opinion that the industry wastes too much money by duplicating clinical trials.

Are you listening, HTA experts?

The UK as it heads to Brexit

Britain's National Health Service (NHS) needs to provide health data to private companies who in turn need to provide the NHS with developed new technologies.

This according to a report by the Kings Fund entitled *Reform: Making NHS Data Work for Everyone*, which also cites the need for better social engagement on data sharing.

There are "no public sector models or examples that allow patients to participate in the conversation", the Kings Fund says.

Meanwhile, the UK's National Institute for Health and Care Excellence has issued new guidelines outlining what developers and regulators should be looking for when they assess digital health technologies, including potential risks.

In other news from Britain it seems that the sharing of health data messages is increasing. Using the so-called NHS Spine system, these "transactions" recently reached around one billion in a single month for the first time.

EAPM

7th Annual Conference

BRUSSELS

8–9 April 2019

4th EAPM SUMMER SCHOOL

19–22 June 2019 // Leuven

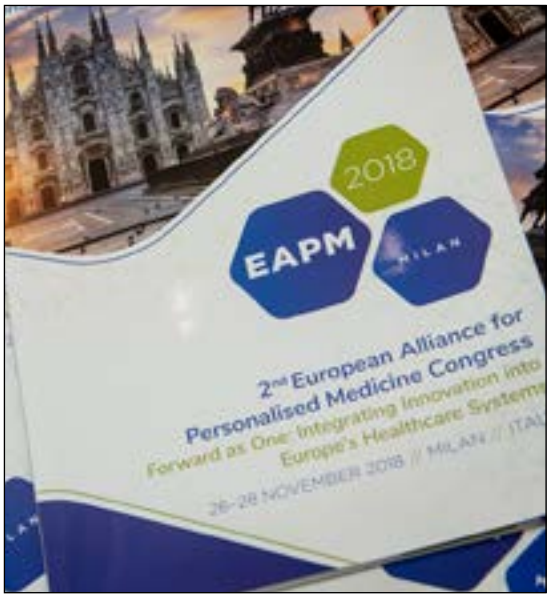
EAPM

3rd Annual Congress

BRUSSELS

18–20 November 2019

Milan Congress, Forward as One



More from Milan...

On a considerable down side for the nation, however, it seems that some 40% of the Britain's cancer patients were originally misdiagnosed, often more than once.

A study also found that 12% of UK patients are paying for some or all of their treatment themselves. This is in order to avoid excessive waiting times or to get treatments that aren't covered by the NHS.

Genomics and data

Ahead of its late-2019 EU presidency, Finland has said it is working on legislation geared towards to creating a national genome centre.

The operation has been delayed since it was first floated in 2016, because the government failed to pass a bill this year in respect of a legal framework for the collection and use of health data.

Meantime, Belgium's Health Minister **Maggie de Block** has been discussing the use of genomic data in healthcare with the public, expressing the view that input from patients and others is crucial when formulating policies, making sure that there is no discrimination based on data, and necessary rules for consent and transparency.

Elsewhere, the Commission is going big on 'Common European data spaces', with a view to making sharing information among EU countries "seamless, while ensuring full compliance with the General Data Protection Regulation".

It plans to launch a data-sharing support centre by mid-2019.

The SPC waiver. Again...

Despite there being the best part of a year before the next Commission moves into the Berlaymont, there can be no dilly-dallying on Parliament and Council reaching an accord on the supplementary protection certificate manufacturing waiver.

Timing is everything, or so the debate runs: Poland wants the waiver to apply from 2021, while Bulgaria, Cyprus, Greece, Lithuania, Portugal, Slovakia, Slovenia and Spain all nodded broad agreement but didn't offer up an exact date.

Medicines for Europe's Legal & External Relations Director, **Sergio Napolitano**, spoke about a "clear political will to reach an agreement within the Council before the end of the year", on the back of this.

Unfortunately, Denmark, the Czech Republic and Sweden think the waiver should only apply to future SPC applications, as does EFPIA, which represents the pharmaceutical industry.

EFPIA's director general **Nathalie Moll** pooh-poohed the notion of applying the waiver to existing property rights in a blog.

She that if that happened there would be a risk of "moving the goal posts for life science investors that have committed to Europe under the existing framework of IP incentives".

Rare diseases

Members of the European Parliament, under the stewardship of MEP **Gesine Meißner**, have been urging the EU to take more action regarding research and access to rare disease treatments.

Meißner said in plenary, mid-December: "We need to improve things here and that's why it's very important that we promote research and we make sure that financing is available."

She said that everyone wants to have a stable state of health and might think that in Europe things are going well.

But she disputed this, pointing out that there are up to 8,000 rare diseases in Europe and with some 30 million affected by them. This is a considerable number, she said.

Meißner said that her father and brother died of a rare disease and there was no medicine or treatment to help them. Her son died of a disease as recently as six years ago - one that was unidentified at the time.

She asked the Commission what measures it had taken so far to ensure accurate and timely diagnosis of rare diseases, as well as access to affordable medicines required to treat them, across the EU?

The German deputy also asked how the Commission promotes research in the field of rare diseases and into the development of new or improved methods for early diagnosis?

Other questions were:

- How does the Commission intend to further these efforts and achieve measurable improved outcomes for rare disease patients in all EU Member States?

- How does it intend to guarantee access to information and to medicines, and medical treatment for rare disease patients throughout the EU?

- How does the Commission promote better coordination and learning across Member States, given that access to early and accurate diagnosis can have a significant impact on the patient's prognosis and quality of life?

On top of these, Meißner asked what are the Commission's plans to ensure sustainable funding of patient organisations supporting the European Reference Networks on rare diseases, for example by means of the European Joint Programme on Rare Diseases?

Vytenis Andriukaitis (pictured), European Commissioner for Health and Food Safety, said that in the past 20 years the Commission has been working on rare diseases, creating networks and encouraging innovative ways of working and research.

He said the Commission had taken concrete steps to ensure that patients are diagnosed as soon as possible. Following the entry into force of the legislation on cross-border healthcare, Member States have put in place national contact points.

These are key to inform patients and doctors on rare diseases and to access the appropriate expertise in the patient's member state or another, he said.

Meanwhile, eHealth service infrastructure is in place and is workable, helping in exchanging views and data across borders.

Commissioner Andriukaitis said that the European Reference Networks (ERNs - launched in March) aid access to timely diagnosis and treatment and help in linking expertise. He praised successful EU-wide cooperation in this area.

On access to medicine, the commissioner said that studies published earlier in 2018 on pharmaceutical incentives drew attention to rare diseases. There will be an additional study that will assess the functioning of the Orphan and Paediatric Regulations before the end of this mandate, he said.

Andriukaitis said that regarding terms of measures to allocate more research to rare diseases, Horizon 2020 and the 7th Framework Programme had more than 200 projects in this area and now they provide in excess of €1 billion dedicated to rare diseases.

Regarding access to information, Commissioner Andriukaitis highlighted the work of the Joint Action on Rare Diseases, which has supported the development and sustainability of the Orphanet database while helping to achieve an appropriate codification of rare diseases in health systems. The database is the biggest repository of information on rare diseases in the world, he said.

Regarding EU legislation on orphan drugs, the Commissioner said that the Union has directed investment in areas that were previously neglected, while emphasising that, if the Commission's plans on health technology assessment come into force, this will help things going forward.

Commissioner Andriukaitis meanwhile pointed out that ERNs are supported by the Commission through the Health Programme and the Connecting Europe Facility programme. They have also been looking into setting up a rare disease joint programme co-fund. This will encourage participation of ERNs.

He also said during the plenary that big data needs big actors. National registers are too weak to address many of these issues on their own. He wanted to encourage Member States to cooperate in this area, as no one country can go it alone.

For her part, MEP **Sirpa Pietikäinen** said that rare diseases are actually not rare, adding that approximately 10% of Europeans have a rare disease. Single Member States, regions and cities do not have all the information needed, which is why European cooperation is required.

She added that timely diagnosis, using the cross-border healthcare initiative and eHealth, are good examples of areas where Europe can help, and hoped that the Horizon programme would further develop the ERNs and have artificial intelligence help doctors diagnosing rare diseases.

The Finnish MEP pointed out that orphan drugs are extremely expensive, and called for a European fund to buy orphan medicines for rare diseases.

Several Parliamentary colleagues had their say, including **Ana Mirand**a (pictured), who described rare diseases as a major public health issue. She also called for funds, cross-border cooperation and ensuring that people get the right treatment as well as better access to orphan drugs.

Of course, this all comes against the backdrop of a limited scope for Commission action given Member State competence over health.

Yet, as almost seven-out-of-ten voters want the EU to intervene more when it comes to healthcare, current and new MEPs, plus the Commission, will have plenty to think about down the line. Many would-be MEPs have already said that they will campaign on a health platform in the run-up to the May vote. Good to know.

In the news

As ever, the Alliance has been busy engaging with the media. Below you can find links to recent articles.

Close-of-the-year event is huge success

'Shoulder to the wheel' time for patient access

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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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