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Regulators face 'challenge' over innovation

Welcome to Day Two of EAPM's Congress '*Personalising Your Health: A Global Imperative*' here in Belfast, Northern Ireland. This is the second of five daily newsletters highlighting what has happened already and what is to come today.

Yesterday at the Waterfront venue featured the opening plenary Presidential session on '*Growth in Personalised Healthcare - The promise for future generations*' during which Lorraine Nolan, of Dublin's Health Products Regulatory Authority, spoke.

'"Innovation is one of the single biggest challenges and one of the biggest opportunities facing regulators, we need to keep abreast of what is happening.

"One of the key mechanisms for doing this is horizon scanning to anticipate new innovations and technologies that allow us to prepare.

"Without this the Authority would not be able to maintain its relevance into the future. If we don't horizon scan we don't stay ahead of the curve and we will become out-dated and unable to maintain relevance into the future."

Congress heard that whether it is possible post-Brexit growth in the UK or resilience in China, improved healthcare supports economic activity.

A healthier Europe will mean citizens spending less and less time in hospitals under expensive treatment regimes, often at a direct cost to the taxpayer, and it will also mean that people receiving the right treatment at the right time are more able to stay in the workplace, thus generating wealth rather than whittling it away.

By the same token, a shift towards preventative medicine will reduce costs still further.

A focus on research into new medicines, innovation and cutting-edge treatments will also create jobs – whether they be in research itself, education, design and manufacture of in-vitro products or within the pharmaceutical industry.

Unfortunately, healthcare innovations are only very slowly being embedded into Europe's healthcare systems. There is a clear need for better focus from policy makers.

Today's highlights at a glance

- **Presidential Session - Research frontiers in personalised medicine**
- **Genomics and Medicine – Crossing the Rubicon**
- **The most dreadful "neglected" cancer: pancreatic cancer**
- **Innovation, Screening, Guidelines: The Case of Lung Cancer**
- **Cancer in Europe : Responding to the Challenge**
- **Big data in hematology: the HARMONY project**
- **Speech by Fidelma Macken, former Justice of the Supreme Court of Ireland, and a judge for several years at the European Court of Justice**
- **Inaugural HI-5 Awards, presented at Titanic Belfast.**

Lorraine Nolan, of Dublin's Health Products Regulatory Authority. Photo by Simon Pugh Photography

The incentives for developing new and effective medicines are currently inadequate. And, as a result, innovation slows and health systems and patients suffer.

It is clear that incentives need to be revised, and it will be the ability and willingness of governments, payers and industry to invest in these new technologies which will determine whether patients will have access to much-needed innovative treatments.

One key to success will be the development of a new reimbursement model, using value-based pricing and taking account of the impact of a treatment on overall health spending, the Congress heard.

Cooperation within the European Commission and Member States in support of EU projects is necessary, as is cooperation with the European Medicines Agency and HTA bodies.

On top of this, a policy bridge is required and a conscious decision among the powers-that-be in Europe needs to find a way to harmonise multiple strands of activity and responsibility in the health arena.

The end goal will be for the EU to more effectively integrate the incredible advances in science into healthcare systems, for the benefit of all patients.

A further session on the future of healthcare heard that if Europe is to grasp the opportunities it incontestably has for making real improvements in the health of European citizens, this haphazard *laisser-faire* attitude will no longer do.

The short-term disconnected approach must be abandoned in favour of a longer-term vision that recognises explicitly that real progress depends on a comprehensive appraisal of needs and a cooperative response to come up with answers.

This is not a plea for relying on a utopian omniscience. Many problems and issues cannot be foreseen, so no longer-term plan will ever have all the answers.

But what a long-term vision can do is to provide a structure, a framework, a consensus on principal objectives, so that as unforeseen issues arise, they can be confronted within an agreed context, and intermediate solutions can be sought that will not conflict with one another or with overall goals.

That would avoid the difficulties Europe encountered in regulating data privacy, for instance. Aiming at the perfectly acceptable objective of protecting citizens from incursions and intrusions and abuse by commercially driven internet services and social networks, the EU unwittingly created rules putting at risk the transfer of personal data that is the lifeblood of medical research.

A longer-term view would also avert knee-jerk reactions and permit legislation to emerge from mature reflection rather than panic.

Special lecture: Alexander Eggermont, Director General, Institute Gustave Roussy, France

Alexander Eggermont, Director-General of the Gustave Roussy Institute, a top cancer institute in Europe, spoke about the revamping of their clinical research infrastructure to adapt to the rising new field of translational research and his ambitious plans for the next five years, in particular, his vision for Cancer Core Europe, positioned to be a virtual European Cancer Institute.

Gustave Roussy is a comprehensive cancer centre, which means it works in the three areas of care, research, and education. It is the largest cancer institute in Europe by volume of activity; seeing up to 12,000 newly diagnosed patients a year.

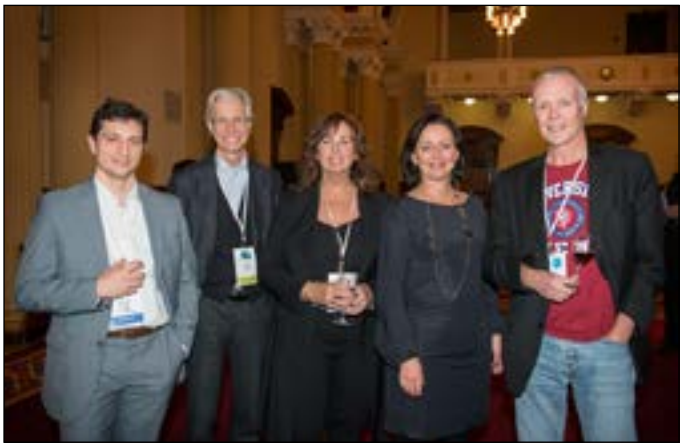
The institute can be seen as the European counterpart of the Memorial Sloan Kettering cancer center in the US.

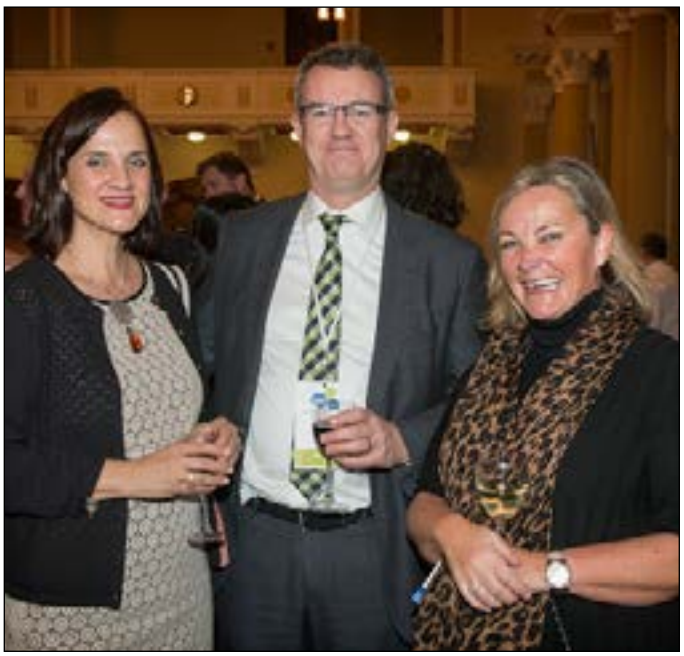
Alexander said that he can "attribute this success to the major changes we have implemented during the past five years".

"Our most significant milestone has been the overhaul of our infrastructure to better reflect and adapt to the paradigm shift that is occurring in clinical research."

A warm welcome to all at Belfast City Hall

Speakers and delegates gathered last night for a reception at Belfast City Hall, featuring an award ceremony and an address by Parliamentary Under Secretary of State for Northern Ireland, Chloe Smith MP (top right). Chloe is pictured again bottom-left with David Boyd of AstraZeneca and Malta's deputy prime minister Chris Fearne. *Pictures by Simon Pugh Photography*





And the winners are...

Last night saw two winners pick up inaugural EAPM awards at a welcome reception in Belfast City Hall.

The EAPM SMART Award (Smaller Member states And Regions Together) went to Malta, while the Patient-centric Innovator Award went to AstraZeneca.

Christopher Fearne, Malta's Deputy Prime Minister, picked up the SMART Award while David Boyd received the AstraZeneca prize from Stephen McMahon, president of the Irish Patient Association.

Before handing over the award, McMahon described the EAPM Congress as "a cornerstone event for personalised medicine in Europe".

During its recent presidency of the EU, Malta supported and acted upon European Council conclusions on strengthening the balance in the pharmaceutical systems in the EU and its Member States. Chris Fearne said he was "touched and delighted with this award".

And in a message, AstraZeneca's Senior Vice-President, Precision Medicine and Genomics, Ruth March referred to "AstraZeneca's scientists who are working tirelessly to develop innovative, targeted medicines for every patient who needs them, and to match those treatments to patients most likely to benefit".

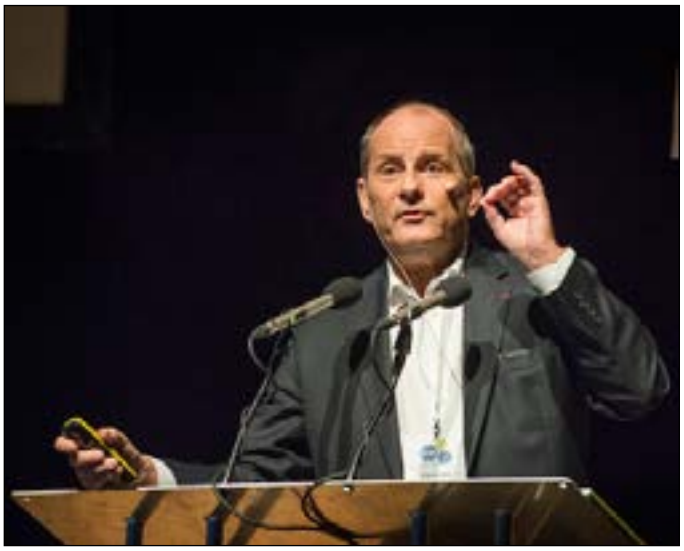
The Parliamentary Under Secretary of State for Northern Ireland, Chloe Smith MP, also spoke during the evening. As part of her speech she said: "It is crucial to have health and wealth across the UK. And Northern Ireland demonstrates a strong collaborative approach and offers a great opportunity in life sciences."

Ms Smith said that, post-Brexit, the UK would continue to work with EU partners in science and innovation, backed by a strong legislative strategy.

The evening was presided over by Gordon McVie who, as well as being a world-renowned oncologist is co-chair of EAPM.

He said: "It is a delight to be here, not only at the start of our first annual Congress held on the back of five presidency conferences, but also to see well-deserved awards given to Malta and AstraZeneca."

"Both of these recipients have, in their own ways, substantially furthered the cause of patient-focused personalised medicine and I know we all wish to congratulate them."



Alexander Eggermont, Director General, Institute Gustave Roussy, France. Photo by Simon Pugh Photography

EAPM's executive director, Denis Horgan, thanked Belfast for the warm welcome, adding that: "The Alliance believes that Europe's health policies need to recognise and tackle the inherent health system vulnerabilities faced, specifically, by smaller countries and in the regions of the larger ones. Well done to Malta for facing this head on, on the ground."

Horgan went on to say that the patient-centric innovator award exists to highlight the role of a company which has made putting patients at the centre of their own healthcare a key element during the course of its own, ongoing innovative work.

He added: "We are happy to note that an EAPM member organisation, the European Cancer Patient Coalition, nominated AstraZeneca for this award. As Gordon said, it is well deserved."

Tonight in Belfast will see five more awards given out. These are the HI-5 (Health Innovation Five) prizes and are also a new development from EAPM.

The first HI-5 award will go to the EU-based Minister who has best supported health innovation in personalised medicine, with the second going to the EU-based region that has done the most to support innovation in that arena.

HI-5 award number three will go to a EU-based research centre for innovation in personalised medicine, the fourth to the best company to promote personalised healthcare, and the final HI-5 to the EU-based hospital doing the most to integrate personalised cancer medicine.

"""""""""""""""""Quotes from the sessions""""""""""""""""""

"Security should not be about preventing health data from being effectively used, but about making good use of those data in a secure way."

Jevgeni Ossinovski,
Minister of Health, Estonia

"Personalised medicine is going to change public health practices whether we like it or not! So we'd better get prepared now."

Natasha Azzopardi Muscat,
Health Information and Research, Malta

"We need a cross-sector collaborative approach to develop healthcare for all, including academia, research and industry to maximise the development and potential of genomic medicine."

Sue Hill,
CSO, NHS

"The Governement issued today the UK Industrial Strategy where genomics is singled out as an important vehicle of growth. This is a good day for genomics."

Sir John Chisholm,
Genomics England

"One of the greatest challenges we have to tackle is the way we manage cross-border data sharing."

Bogi Eliasen,
Copenhagen Institute for Future Studies

"Cultural gaps are holding back translation of research outputs into marketable products."

Richard Barker,
CASMI, Oxford

Patient story - pancreatic cancer

Londoner Tom, aged 48, ignored what turned out to be early symptoms of abdominal pain and weight loss, as they did not clearly point to pancreatic cancer. He later developed jaundice, however.

It became clear to Tom that one of the major issues with the disease is that the symptoms can often be vague, and that by the time a patient is diagnosed the cancer may have spread to other organs and even into bones.

This was the case with Tom and, before he died, he was adamant that there needs to be more dedicated support and information available for people with the disease.

Carin Smand, European Hematology Association. Photo by Simon Pugh Photography

Earlier today

Presidential session: Big Data Saves Lives

In respect of personalised medicine, Big Data represents the vast and continuously growing amount of health information (including biomedical and environmental) and its usage to drive innovation in translational research and health outcomes tailored to the individual.

Using these data to first understand the cause of disease, the medical profession can then develop new drugs and therapies to find the cure, as well as other health interventions targeting the individual.

The personalised, individual approach requires advanced technologies and processes to collect, manage and analyse the information and, even more importantly, to contextualise it, integrate it, interpret it and provide rapid and precise decision support in a clinical and public health context.

Not only does Big Data offer the potential to revolutionise the effectiveness of health interventions, it may also help ensure the more effective management of resources in what are increasingly cash-strapped public healthcare systems.

Big data in healthcare is being used to predict epidemics, cure disease, improve quality of life and avoid preventable deaths.

With the world's population increasing and everyone living longer, models of treatment delivery are rapidly changing, and many of the decisions behind those changes are being driven by data.

The drive now is to understand as much about a patient as possible, as early in their life as possible - hopefully picking up warning signs of serious illness at an early enough stage that treatment is far more simple (and less expensive) than if it had not been spotted until later.

This, then, was the theme of the session at the Congress, featuring chairman Mark Lawler (Translational Cancer Genomics, Queen's University Belfast), Julia Wilson (associate director, Welcome Trust Sanger Centre, Cambridge), Andrew Morris (director, Health Data Research UK), Ewan Birney (director, European VBioinformatics Institute, Cambridge), Mene Pangalos (executive vice president, Innovative Medicines and Early Development Biotech UNit, Cambridge), Ain Aaviksoo (deputy secretary general for e-services and innovation, Estonia), Ernst Hafen (ETH Zurich) and Margaret Grayson (chairwoman, Northern Ireland Cancer Research Consumer Forum, Belfast).

Ewan Birney was the first to praise and congratulate his follow workers around the world: "The bulk of our work [in genomics deployed standards] is done by tactical volunteers across the world. But there is still much to do, particularly concerning policy, and the regulation and implementation of science, and this is where Big Data truly comes into its own."

Margaret Grayson then warned: "However, the implementation of science is frequently like a post-code lottery, as to whether patients will receive the correct tests or not."

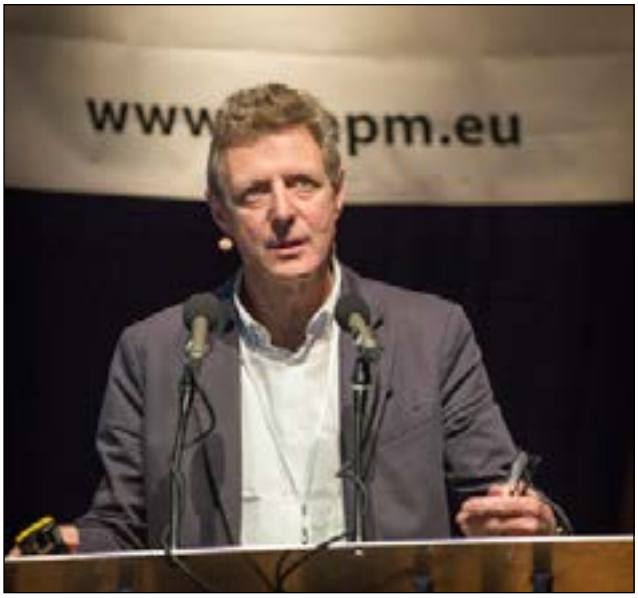
And Ernst Hafen added: "With my strong interest in human genetics and personalised medicine, I believe that an individual's control over his or her personal health data will be a key asset for better and more effective healthcare, and the growth of big data posits legal, ethical and societal issues about health data ownership. It is very important to find commercial models permitting owners, not third parties, to benefit from personal data assets."

Core aims include:

- *The integration of personalised medicine into clinical practice will be enabled through scientific evidence generated by multiple data*

- *Complex data protection rules are a major regulatory constraint and an impediment to biomedical progress toward achieving personalised medicine. Updating these EU rules should take account of the impacts for individual's health and global healthcare*

- *Changes are needed in the way data are collected. New technologies are revolutionising the possibilities for capturing data. The use of electronic medical record data as a source of readily available research data eliminates the need for costly and lengthy active new recruitment of trial subjects, and*



Ernst Hafen, ETH Zurich. Photo by Simon Pugh Photography

drastically reduces collection of redundant specimen and data.

- *In order to allow integration of information from multiple sources, the data must be well characterised, standardised, and compatible*

Eyes do not lie – you are what you can see! Options for personalised medicine in eye diseases

The battle against eye disease in Europe needs to be fought at EU level. There are some 39 million blind people in the world, but 80 per cent of blindness can be cured or prevented. That's 31.2 million people who are blind when they needn't be.

Studies suggest that eye disease costs society in Europe some

Key aims are:

- *More timely diagnosis, intervention and, at the core, research and awareness of the extent of the problem are key*
- *Research into the causes of cataracts and other eye diseases needs boosting across the EU, with platforms put in place for effective collaboration between academia, industry and healthcare systems*
- *There is a definite case for more screening programmes for preventable blindness, coupled with a need for agreement and coordination across the European Union's Member States on this*

Coming up later

Presidential Session: Research Frontiers in Personalised Medicine

Europe's and the international research landscape remains too fragmented. A lack of critical mass in many research centres means not enough patients, biological materials, technological resources or competences.

Wider collaboration and better infrastructure would help: technical platforms for genomics and other specialty disciplines, screening facilities for new pharmaceutical agents, biobanks for tissues and biofluids, plus quality-assured patient registries.

So too would better resources, for prospective validation of biomarkers that may be predictive for treatment, networks on biostatistics, epidemiology and outcomes research.

Integration along the research continuum would make it easier to bridge basic/preclinical research and clinical research in early translational research.

Core aims include:

- *Research needs to be structured to emphasise the key position of the patient in personalised medicine*
- *Research needs to span over the entire translational chain. Healthcare professionals need to be able to translate research into clinical practice and bring it forward to public health decision makers*
- *There is a need for a cross-sectoral research involving pre-clinical, clinical work, academia, industry and patients, to be completed by real world data.*
- *Patients have to be involved from the very beginning of research projects*
- *New forms of collaboration are required between academic centres, the pharmaceutical industry, regulators and payers*
- *Open research collaboration mechanisms are required for translation from basic research to clinical research, including access to data and diagnostics which allow patient stratification*

The most dreadful "neglected" cancer: pancreatic cancer

The battle against pancreatic cancer needs to be fought at the highest level, having arguably the lowest survival rate of any cancer. There were an estimated 103,773 new pancreatic cancer cases in 2012 in Europe, making it the 6th leading cause of cancer-related death, with 104,481 estimated deaths in that year.

And according to predictions across the EU, mortality of pancreatic cancer will be up by 4% in men and 5% in women since 2009.

Ewan Birney, European Bioinformatics Institute. Simon Pugh Photography

Denis Lacombe, director general of EORTC. Photo by Simon Pugh Photography

This 'silent' disease does not cause identifiable symtoms at an early stage and is, therefore, currently hard to detect. By the time symptoms appear, the cancer is often already advanced and it is too late for surgery in many cases.

The overall five-year survival rate in Europe is 6%, although outcomes are slightly better for the small percentage of patients whose disease is discovered early.

To address these issues and lower the immense burden on society, it is necessary to develop a comprehensive pancreatic cancer research community and provide the tools and resources this community needs in order to make scientific breakthroughs.

This will clearly require increased investment in pancreatic cancer research through Member State and EU public funding programmes, as well as in the private sector, through NGOs, industry and others.

Key aims include:

- *Budget planning is required for pancreatic cancer. National cancer plans need to prioritise funding for treatment, based on dialogue between the medical professions, commercial and academic research, and paying agencies*

- *Research into pancreatic cancer needs boosting, with platforms for effective collaboration between academia, industry and healthcare system.*

- *The European Commission should allocate long-term funds for research into pancreatic cancer, with increased investment in research centres, and with advanced databases of biological and clinical data and clinical databases.*

- *Patient registries and databases are essential to permit the pooling of data needed to achieve a sufficient sample size for clinical research. Regulations need to be adapted to allow more sharing of patient data and biological materials*

- *Improved coordination of clinical trials - and of recruitment - is needed, with better access to information for researchers, doctors and patients. strategy, and a common approach to mobilising and integrating scarce and scattered resources*

Innovation, Screening, Guidelines: The Case of Lung Cancer

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

There is a need for agreement and coordination across the European Union's Member States.

Of course, cost-effectiveness questions arise whenever population-wide screening is considered, especially in relation to frequency and duration. Yet, the potential benefit of low-dose CT lung cancer screening would almost certainly see an improvement in the lung cancer mortality rate in Europe.

Findings in both Europe and the US strongly suggest that lung cancer screening works, although debate continues about the best way to implement screening of this kind, and even how to properly evaluate 'cost effectiveness' - who should decide?

Guidelines could help to tether costs, by bringing in improvements to the efficiency of screening methodologies and, thus, programmes themselves.

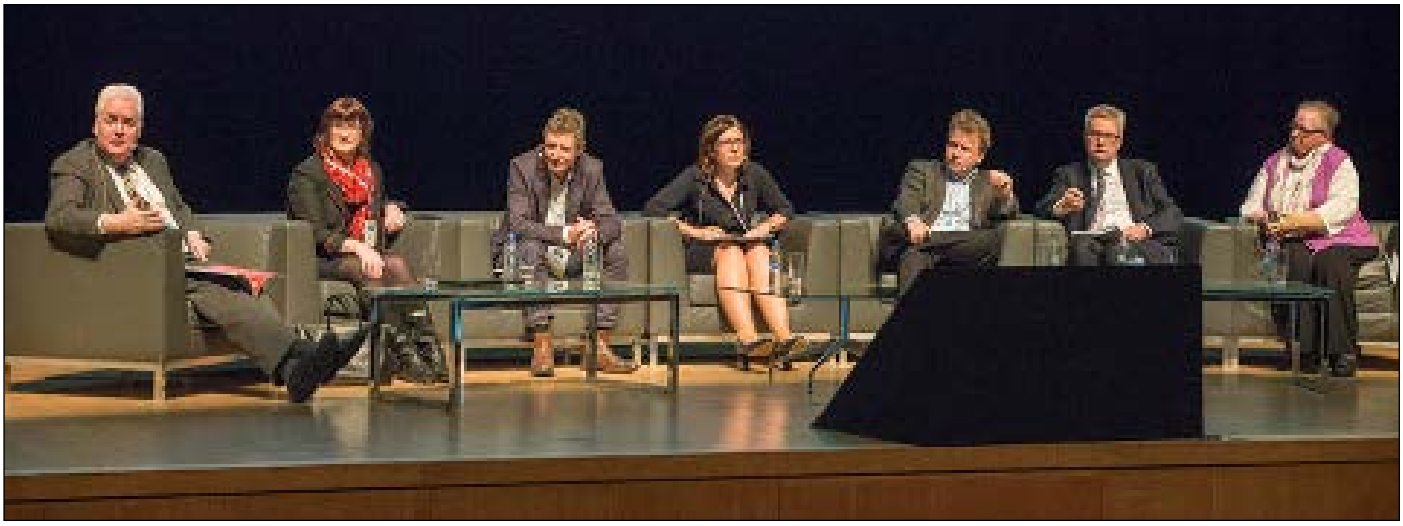
Key to such a situation would be making the best use of efficient risk-assessment methods, top-of-the-range imaging technology, and guidelines that encourage the minimisation of invasive procedures and risk to the patient.

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*



Panel for the session on Big Data. Photo by Simon Pugh Photography

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

Big data in hematology; accelerating more efficient drug development, regulatory evaluation, access appraisal and treatment strategies (HARMONY)

The HARMONY project is a European Network of Excellence that captures, integrates, analyses and harmonises Big Data.

The consortium is made up of 51 partners: 44 participants from 10 European countries and seven pharmaceutical companies from EFPIA. It brings together key stakeholders in the clinical, academic, patients, HTA, regulatory, economic, ethical, ICT, and pharmaceutical fields.

HARMONY uses high-quality, multidisciplinary sources to acquire valuable knowledge across the spectrum of haematological malignancies (known as HMs). The goal is to unlock valuable knowledge on HMs.

Figures suggest that healthcare costs for each patient with blood cancers reach twice the figure compared to average cancer costs. This is primarily due to the need for longer time spent in hospital coupled with more complex treatment and diagnosis. The total cost of blood disorders to the European economy was in the region of €23 billion in 2012.

Blood cancers are in the top ten of the most common forms of cancer and are responsible for approximately 100,000 deaths in Europe every year. The proportion of healthcare cost within the total economic burden is higher for malignant blood disorders than for other solid tumours.

There are, therefore, very strong arguments in favour of raising public awareness about the effect of blood disorders in Europe, given that malignant blood disorders represent a leading cause of death, healthcare service use and costs.

Key needs include:

- *A pan-European approach to the development of new tools to refine HM outcome definitions*
- *There is a need to establish a clinical data-sharing platform that empowers clinicians, patients and policy stakeholders to improve decision-making procedures and identify appropriate treatments to patients with HMs*
- *Europe must create a community that reflects the HM landscape made up of key expert academic institutions, national clinical disease networks, and European organisations. This should include the active involvement of patient advocacy groups, clinicians, the pharmaceutical industry, regulatory agencies and other stakeholders*
- *Provision of tools for analysing complex data sets comprised of different layers of information so that molecular and clinical data can be linked to predict clinical outcomes*
- *Identifying specific biomarkers, which better define outcome parameters*
- *Providing a framework for legal, ethical and governance issues*

Richard Gallon attending the Congress. Photo by Simon Pugh Photography

Twitter hashtag for this Congress:
@eapm2017

European Alliance for Personalised Medicine

In close collaboration with our partners:

Platinum partners:

Gold partners:

Silver partners:

Bronze partners:

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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