MEETING THE CHALLENGE

A PARLIAMENT MAGAZINE SPECIAL SUPPLEMENT ON PERSONALISED MEDICINE

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POLITICS, POLICY AND PEOPLE

DODS

IN ASSOCIATION WITH European Alliance for Personalised Medicine

Meeting the challenge

Introduction by David Byrne and Helmut Brand

Healthcare is rapidly moving into a new phase where innovative technology can provide detailed information on patients' individual biological characteristics. This data and its interpretation will form the basis of a new, personalised medicine. This innovative approach can be defined as 'the right treatment, for the right patient, at the right time'. This seemingly simple idea, however, is supported by incredible advances in the scientific understanding and treatment of disease. It is no longer accurate to put patients into large groups based on outmoded definitions of disease. The growing subdivision of patient populations into smaller and smaller disease groups necessitates a sea change in the techniques and understanding that inform our healthcare system. Personalised medicine brings with it the possibility of smarter, better and more cost effective healthcare that allows for the more accurate prevention and treatment of disease.

This brave new world of personalised healthcare will require significant change across a variety of fields. Patients must be engaged with and informed if they are to reap the benefits of these new medical methodologies, new ICT tools must be made available and accessible, while healthcare professionals must receive the education and training needed to deliver the best care possible.

Access to information will be a vital element in the new medical paradigm, and legislation will be needed to support healthcare professionals and researchers who require vast reams of data to fuel the better prevention and treatment of disease. Old ideas regarding the risks and benefits of medicines must be revised and revamped if the true added value of personalised medicine is to be realised. And the development of new treatments and diagnostics must take account of the potential and peculiarities of -omics technologies. The pace of transition from research to actual patient benefit must also be accelerated.

These complex challenges require an ambitious and equally complex response. A transformed healthcare system that delivers the best care for patients, puts research and innovation at their service, and gives the best possible value can be achieved, but the right conditions must be in place. Policymakers, stakeholders, patients and healthcare professionals can all play a role.

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Safety first?

Balancing the individual's right to privacy with society's healthcare needs is vital for continuing progress in medical science

Improving knowledge of medical conditions and their treatment is directly reliant on the open sharing of personal medical data. However, this places the many benefits of shared health data against rising concerns over the privacy of information. In an age where vast reams of data are being collected on almost every aspect of citizens' lives, the defence of fundamental rights is paramount, but, with the importance of personal health information expected to rise yet further, a balance must be struck between innovation and security.

With the European parliament continuing to discuss its gargantuan data protection regulation, there are concerns that EU legislation in this area could hamper the development of personalised medicine. President of Sage Bionetworks Stephen Friend warned that EU rules are already "preventing innovation", adding that, while regulations might ensure the safety of data, Europe will not be "part of the solution" when advancing healthcare. Friend also stressed that the EU must avoid moving to a situation where Europe is "unable to host research activities because its rules are too restrictive".

The engagement and involvement of all stakeholders is vital if the balance between the diverging priorities of improving healthcare systems and protecting patients' rights to control the use of their data is to be found. Kaisa Immonen-Charalambous, senior policy advisor for the European Patients' Forum, underlined the importance of an inclusive approach by saying that patients are happy for their data to be used to help others if they "know what it will be used for and that it will be secure". "I urge member states, MEPs and the European commission to take patients' views into account", she said. While, she said that patients that are informed and reassured about the use of their data are willing and enthusiastic for it to be used to help others, she warned that enforcing explicit documented consent could "overburden medical services".

Paolo G. Casali, from the European Society for Medical Oncology, however, said that the principles of patient consent must be updated to match technological advances in healthcare. For him, the use of data and tissue samples outside of trials or beyond their end should be made possible 'without re-consent' if the patients gave their broad consent for the initial use of their data. The reasoning behind this is to ensure that medical research, for example for cancer, is allowed to progress in a reasonable manner, without having to go back to a patient multiple times to get their consent. Notably, since any research should be approved by a reviewing body, such as an independent ethics committee, it would ensure that ethical requirements would be fulfilled and respected. Most importantly, broad consent is consent to all effects, including the right not to give it, or to withdraw it at any time. Casali also highlighted that the 'numerous member state regulations' currently in force represent stumbling blocks to data sharing. He noted that patients 'cannot donate' their data in several countries, and he called for a pan-European framework that supports patients' desire to share their information.For Irish MEP Nessa Childers, the dangers are clear. "If personalised medicine is to succeed", policymakers must ensure data protection regulations facilitate the sharing of medical information as too much focus on "safety can sometimes mean no solutions".

Stakeholder perspective

Prof. Richard Frackowiak, of ScienceEurope, says that the EU must be clearer on what kind of data regulation it wants. The European commission's draft regulation on data privacy suggests that no data can be used for medical research without explicit consent. This is clearly impossible for studies involving large numbers of people, especially if the analyses proposed are retrospective. A rational legal framework is needed that allows research which benefits populations while protecting individual privacy. Article 83 of the regulation – which sets out conditions for processing personal data for medical research – should be modified to introduce the right level of proportionality between data usage and explicit consent. The experience of such regulation through ethics committees, which has been very successful, should be made use of.

The right tools?

ICT tools are set to transform the clinical process for healthcare professionals and patients alike

New and innovative technologies will form a crucial part of the success of personalised medicine, but these new medical models will require the collection and interpretation of vast reams of high quality and standardised data. With patients the centre of the new medicine, their involvement in this process is crucial.

Web communications senior manager for Eurordis Denis Costello said that there is a potential "tidal wave of personalised medicine" on the horizon. Costello stressed that the success of this new personalised approach is dependent on "Putting ICT tools into the hands of patients and families so they can make a difference." "We need to work out what kinds of ICT tools we can put in the hands of patients to help them actually participate in the personalised medicine revolution," he added. Costello highlighted the growing popularity of "health apps on smartphones and tablets", saying, "These tools can really help patients report and self-track how they manage their own disease-complementary data sets – particularly in the rare disease area where no natural histories often exist for specific diseases."

With patients beginning to collect their own data and use technology to feed this back into the clinical process, professor consultant for the European Science Foundation Kirsten Steinhausen called for policymakers and stakeholders to tackle the "data handling issue". "We need new models and decision making processes that use a personalised medicine approach," she said. "We must ensure that ICT solutions support healthcare professionals and patients and provide access to treatment and prevention options." She called for "ICT interfaces to be developed for citizens", stressing that the "heart of this is the patient" and that there is a need for "more support, funding and better infrastructures if personalised medicine is to become a reality". "The huge volume of data storage and transfer needed to carry out the personalised medicine model is a challenge we must face," she added.

There will also be an increasing need to learn how to extract meaningful information from data and read it correctly, Bonnie Wolff-Boenisch, head of research affairs at Science Europe, believes. Besides its use, the filtering of important and (not yet) relevant data, the protection of citizens' personal data will be essential to keep science trustful, she says, adding that, without the right balance, personalised medicine will not get the endorsement of policymakers and citizens necessary for its implementation.

Angela Brand, from Maastricht university's institute for public health genomics, underlined the need for "trustworthy ICT processes", calling for "more honesty with citizens over data protection issues". "There must be trust in the technologies, and this is an issue that must be dealt with by government." She also stressed that any technology-based solutions must go "hand-in-hand with human resource, including improvements in health literacy". "There is an increasing willingness for people to contribute to data practices and use technology," she said, adding that this was being matched by "an increase in demand for a participatory approach, allowing people to manage their own health".

Member of the Portuguese parliament Ricardo Baptista Leite said there "must be more work on informed consent". "In Europe, we have a great variety of tests. Variety leads to questions over whether consent can be truly given with the vast array of options available. We need high ethical standards in order to ensure we have public trust."

President of Sage Bionetworks Stephen Friend underlined the need to prepare for significant changes in the way the clinical process operates, saying, "ICT tools are going to be more fluid, less top-down and everyone is going to be using them."

Stakeholder perspective

Director of Oracle Health Sciences Joel Haspel says that novel technologies can play an essential role in realising the vision of personalised medicine.

"With people born today having a good chance of living beyond 100 years and with their overall health and quality of life being impacted by their genomics, medical treatment, lifestyle and environment it is essential to put the patient at the centre of personalised medicine. Today, Oracle is already working to integrate clinical and genomic patient data for research and care and with the explosion of mobile applications the inclusion of lifestyle data is not far off.

It's new technologies that are already making personalised medicine a reality and innovative, affordable technologies will be required to educate and engage with citizens. These new forms of technology are based on large volumes of data, with a subtle but important shift being undertaken in the type of data used. In simplest terms 'big data' refers to the growing amount of information we have to analyse a disease while 'long data' refers to the accumulated data, integrated from many sources over a person's lifetime. Both are important to the discovery of cures, treatment and prevention of disease.

As a result of this reliance on personal information, data protection will be a big issue because it is highly visible and affects everyone, but we already have technology that can secure data, track data access and de-identify it for broader use."

Quality control

Personalised medicine is reliant on accurate and dependable diagnostics and regulators must take measures to support this undervalued discipline

The right treatment for the right patient at the right time is the central mantra of personalised medicine, a medical discipline in which the crucial role of diagnostics and imaging must be recognised. Their potentially significant impact on the efficacy of therapies, cost-effectiveness of healthcare systems and patient outcomes is pivotal for the future of public health and must be acknowledged by policymakers and regulatory professionals alike.

As Tom Lillie, Oncology International therapeutic head at Amgen, said, the current framework for companion diagnostics is insufficiently coordinated on a Europe-wide scale. In the context of the missing – or fragmented – pieces, including the lack of quality control processes around diagnostics and the different views presented by regulatory authorities on what the right level of clinical evidence is for a companion diagnostic, the European commission proposal for a regulation on in vitro diagnostic medical devices should be seen as an opportunity for improvement. "We must ensure reliability, validity and accuracy," Lillie emphasised. Lillie's request may seem like a reasonable and relatively easily achievable one, but with clarity around standards missing, the current reality is a situation where different labs come up with different results despite using the same samples and reagents – an obstacle that will have to be overcome in order to achieve the clinical and scientific evidence needed before a companion diagnostic can enter into use. To achieve this, a regulatory structure that clarifies the role of the entities involved in the approval process, including the European Medicines Agency (EMA) or its national equivalent, notified bodies, and reference labs, should be created, both to guard against double regulation and ensure that serious delays in patient's access to treatment are avoided. Ian Watson, president of the European Federation of Clinical Chemistry and Laboratory Medicine noted considerable variation within and between nations in the harmonisation of markers' clinical use and their analytical standards, which demand consistency to enable optimal patient care and safety.

Beyond the need for cooperation between regulatory authorities and, ideally, coordination between diagnostic and pharmaceutical development, there is another point on the route to market requiring a joint effort – biobanks. The rising importance of these biorepositories in personalised medicine is such that Guy Frija, president of the European Society of Radiology, called for a specific European programme to be launched to focus on the appropriate structuring of biobanks, with a particular emphasis on interoperability and imaging. Here, Frija sees collaboration between academia, industry and regulators as key.

> *"Diagnostics is a very underrated part of personalised medicine"*
> *Rebecca Jungwirth*

John Crown, consultant medical oncologist at St Vincent's University Hospital, also highlights the pivotal role biobanks play in terms of researching and discovering new biomarkers, making the possibility of reusing samples central, particularly through posthumous tissue analysis. He insists that this option must remain within the realm of possibility under the planned revisions of the EU's clinical trials and data protection directives. The pending regulatory changes carry much potential for companion diagnostics specifically and personalised medicine at large, but even with their shadow hovering over the sector, the biggest obstacle facing companion diagnostics is actually a tendency to overlook them altogether.

As Rebecca Jungwirth, of Roche, said, "Diagnostics is a very underrated part of personalised medicine, people don't understand it well and this must change".

Imaging, a key ally for personalised medicine

Medical imaging is often neglected in the context of personalised medicine and its role is frequently underestimated by policymakers. This is due to the common approach to personalised medicine, which up until now has focused on the different aspects of '-omics', and because the importance of the 'right location' and 'phenotype' is often undervalued within the paradigm of personalised medicine.

Medical imaging has always involved a personalised approach as it assesses the location and morphology of an abnormality, the extent of disease and the involvement of adjacent structures in an individual, which could not be obtained by other means. There is also great potential in its combination with quantitative imaging biomarkers, which can characterise perfusion, blood flow, metabolism and cellularity. Successful translation into clinical applications would allow for the stratification of patients into different risk groups and individualised decisions on prevention and treatment. Radiogenomics is another emerging field that creates a link between molecular diagnostics and diagnostic imaging. As in case of companion diagnostics, clarity around standards is also missing for imaging biomarkers. Differences in imaging modalities and user experiences are important standardisation issues. Before imaging biomarkers can be widely adopted, measures for standardisation and quality assurance must be implemented.

The European Society of Radiology emphasises that it is essential that medical imaging be considered a key component of personalised medicine. In order to reach its full potential it needs dedicated funding and support. This would benefit patients by allowing for development of imaging biomarkers and the implementation of personalised imaging across European healthcare systems.

Guy Frija is president of the ESR

Hans-Ulrich Kauczor is chair of the ESR research committee

Patient power

For personalised medicine, as with all areas of healthcare, the knowledge and empowerment of the patient can be crucial to the success of treatment

Personalised medicine offers tremendous hope and is undoubtedly the next evolution in healthcare, however, for personalised medicine to succeed and for healthcare innovations to fulfil their true potential, an informed, engaged and empowered patient is vital. President of the European Brain Council Mary G Baker stresses that "personalised medicnie starts with the patient".Yet the truth is that nearly two out of three people in European countries have no awareness of personalised medicine – despite the fact that this evolving discipline has vast implications for healthcare issues and personal health. These statistics were brought to light by findings of the PACE cancer perception index: A six-nation, public opinion survey of cancer knowledge and attitudes, involving more than 4300 people.

The findings from the survey were presented by Gary L. Geipel, global oncology corporate affairs senior director for Eli Lilly & Company, at the European Alliance for Personalised Medicine conference in Dublin, March 2013. "The PACE cancer perception index devoted a considerable amount of time to the subject of personalised medicine, and what we found was both surprising and promising," said Geipel. "While only one-third of respondents were aware of personalised medicine, the majority were supportive once the concept was introduced," he said. "The public wants more information on personalised medicine and believe doctors need to discuss its potential with every cancer patient."

"Personalised medicine needs informed and engaged patients, and this is particularly key for life threatening diseases"
Ilaria Cutica

Geipel also highlighted findings that showed people not only recognised the benefits of personalised medicine for themselves and society, but also expressed a willingness to be tested even if it may not work for them.

He countered the "wrong assumptions" that patients are sceptical about sharing their medical records for the benefit of research or taking part directly in clinical research through trials. "Findings from the survey show that there is a readiness among the general public to share medical records and test-results with doctors and scientists for the benefit of research," said Geipel. "Similarly, nearly three out of four people surveyed worldwide said patients need more opportunities to participate in clinical trials of new medicines. Large majorities said that they personally would be willing to participate if a trial offered the hope of life-extending treatment or insights to help future patients," Geipel said.

"Simply providing patients with information, however, will not solve all problems related to improving access to personalised medicine," said Ilaria Cutica, a researcher in general psychology at the University of Milan. "Cognitive and psychological factors impact patient

The *PACE Cancer Perception Index* survey was conducted by PACE (Patient Access to Cancer care Excellence). This Lilly Oncology initiative aims to: encourage public policies and healthcare decisions that speed the development of new medicines; assure cancer treatments respond to the needs and qualities of individual patients; and improve patient access to the most effective cancer medicines. The *PACE Cancer Perception Index* polled more than 4300 individuals from the United Kingdom, France, Germany, Italy, Japan and the United States. To learn more about PACE, visit www.PACENetwork.com.

engagement and involvement, as well as the success of the whole healthcare process," she said. "Personalised medicine needs informed and engaged patients, and this is particularly key for life-threatening diseases." Cutica stressed the need for an assessment of patients' literacy, adding that patients should be given the opportunity to communicate their own personal health preferences, as not everyone wants to be fully informed about their disease. "Patient characteristics must be taken into account and shared in the context of the patient-doctor relationship," she said, underlining the need for a good information flow that takes health literacy levels into account.

That health literacy needs to be a primary consideration was echoed by Donal Buggy, head of services for the Irish Cancer Society, who stressed literacy levels can have a strong "effect on health status". Buggy underlined the need for clinicians to avoid professional jargon and "automatically assume low literacy" when dealing with patients and caregivers. "All healthcare literature should be literacy proofed," he added, before underlining the need to optimise the health literacy of populations if personalised medicine is to be successful.

Pamela Logan, director of pharmacy services at the Irish Pharmacy Union, said that pharmacists must also be engaged in improving and understanding patients' health literacy, particularly in light of a strategy from the Irish government saying that 95 per cent of healthcare should take place at the lowest level of complexity. "Pharmacists discuss therapy doses with patients," Logan said. "Testing patients is crucial for administering correct drug doses," she said, adding that, with patients often discussing treatment with friends, it is important that pharmacists explain the tailored nature of personalised medicines. "We need an efficient communication pathway about testing between doctors, pharmacists, and patients," she noted, stressing that it must be the "right treatment, for the right patient, at the right time."

Irish Health Minister James Reilly also said that it is "critically important to empower patients." Reilly highlighted the high number of hospitalisations when people could be treated at home, and criticised the high level of treatment administered by doctors, "when nurses could do it. " Reilly added, "We in the industry are not the know-it-alls that we think we are – patients must be advised to make their own decisions."

Stakeholder perspective

Birgitte Grube, president of the European oncology nursing society (EONS), says that "educating nurses to meet patients' health literacy needs" could have a substantial impact on the effectiveness of personalised medicine. "While many think that it is the nurse's role to translate difficult medical words and knowledge, physicians could also make a greater effort in this area. It is not just information that must be passed on; we also have to help patients understand compliance and adherence. Disadvantaged groups need particular help with communication and information as this is generally made for people with the same level of education as the person who produces it. Disadvantaged people and older generations are part of society and we must make sure they are able to follow their treatments well. We've sent some nurses on a one-week master class in communication training that involves role play as a troubled patient. However, this is not enough on its own, and when we link with social workers and invite them to the wards to experience treatment, we see positive results."

Trial and error?

Bringing Europe's clinical trial methods and support structures up to speed with modern medical paradigms is vital for the future of healthcare

With healthcare moving into a new phase of understanding of disease and its treatment, it is clear that current methods underpinning the development of new medicines must also integrate these concepts.

Personalised medicine requires "first and foremost an enormous and on-going amount of research", said Irish health minister James Reilly, who added that "research can speed up the translation of scientific advances into benefits for patients, for healthcare and for the economy". Personalised medicine, however, cannot be supported by antiquated trial methods which do not adequately capture the kind of data required for its new methodologies. The advances in stratified diagnosis that are identifying patient subgroups within wider disease categories must be incorporated within modern clinical trials

Director of the European organisation for research and treatment Denis Lacombe said, "Our healthcare is heading for a profound change of its ecosystem." Under the modern medical paradigm "logistics are much more complex" when attempting to gather useful data from clinical trials. If trials continue to be done "in the same way", and don't "integrate translational research methods into their design" then their attrition rate will be high, he added.

Hans V. Westerhoff, from the universities of Manchester and Amsterdam, highlighted "virtual twins" as the "next most valuable phase of personalised medicine". This technique is based on the integration of vast amounts of data (including multiple new biomarkers) by creating "a computer replica for every individual human" which can then have virtual trials run on it. It mobilises the immense powers of functional genomics, ICT and health-and-disease experience of millions of physicians and patients, to compute the treatment that is optimal for each individual patient. The existing format of statistical clinical trials that produces average non-personalised treatments, should be replaced with large numbers of such virtual-twin prediction trials where treatments will differ between individuals, yet the whole procedure is tested relentlessly. Life-science investigators are eager to help, but need assistance from patients, physicians, industry and policymakers. Pfizer's senior director of clinical sciences Miguel Orri said that the "technical tools are available to conduct clinical trials differently", but that currently "costs and complexity are a barrier to sustainable innovation". "It would be nice to get some supporting legislation for virtual trials," he added. Lacombe echoed Orri's call for legislation, highlighting the need for "efficient regulatory processes" that can support the "rapid endorsement of emerging biomarkers" by reducing administrative burdens. "We must push for an efficient EU framework for translation-based clinical research and screening programmes. We need a simplification of European procedures," he added. Lacombe also called for "core public funding for regulatory research", highlighting public private partnerships and "shared costs models", such as the Innovative Medicines Initiative, as key aspects for the future success of European clinical trials.

> *"We must push for an efficient EU framework for translational-based clinical research and screening programmes"*
> *Denis Lacombe*

European public affairs director for Eurordis Flaminia Macchia said that more and more of the products being tested in clinical trials are "genomics" but the proposed revision of the EU's clinical trials directive "doesn't mention this specific aspect". "It would help if relevant knowledge was being gathered," said Macchia, who called for the creation of an "EU database containing information on ongoing trials".

Irish MEP Marian Harkin underlined the importance of having a "proper regulatory framework in place to ensure patient safety and also to ensure that we get the best deal on personalised medicine for EU citizens". It

Stakeholder perspective

Lithuanian MP Juras Pozela says that the Lithuanian EU council presidency plans to put clinical trials and medical devices high on the political agenda. "There are many legal aspects of clinical trials and in Lithuania we face conservative laws that make treatments and clinical trials extremely hard to conduct. My country has the infrastructure and the education, this is not a problem, but we still struggle to run these trials and develop new treatments. My duty on Lithuania's national health committee is to bring clinical trials and medical treatments back to my country. However, EU legislation is moving towards this conservative Lithuanian style. We live in a globalised world and we do not hold a leading position; medical devices and clinical trials must be a priority. We need common European legislation as much as possible, but it must be the right legislation. It is no good to have separate rules in every member state and we must unite the potential of our scientists; only then will we have the results we need. With Lithuania set to assume the EU council presidency, our country's experience will feed well into the EU level debate as we have direct experience of what happens when innovation isn't given the freedom it needs."

Under assessment

Europe's health technology assessment procedures and payers' models must be adapted to ensure timely and efficient access to personalised medicine for patients

If the vision of personalised medicine is to be realised, there must be radical adjustments to the funding and assessment procedures that support access to innovative treatments. The potential represented by these treatments could be limited by weaknesses in health technology assessment (HTA) methodologies. HTA is a tool relying on evidence-based medicine that is used to inform reimbursement decisions based on value for money compared to existing standards of care.

In theory, HTA "should be a good thing for personalised medicine since it recognises and accounts for its superior clinical benefit", says Elena Nicod, research officer at LSE Health. However, what should be a tool to "identify the greater value of a personalised medicine" may struggle to understand the specificities required to assess the contributions of medicines based on -omics technologies. Nicod highlights the issue of a "reliance on randomised controlled trials (RCTs) as the golden standard, and, given the delayed identification of crucial genetic variants and the small patient populations intended for these treatments, there is a greater risk that these RCTs are further split into subgroup analyses which may not be considered sufficiently robust in demonstrating its clinical benefit". Nicod also underlines the need for recognition of the "ethical issues around early access" to drugs for patients where RCTs produced "positive interim results" and were unblinded, allowing the placebo arm to also benefit from the treatment. In these cases, the treatment's benefit relies on these intermediate results, which likely don't show its real clinical benefit.

Alessandra Ferrario, Research Officer at LSE Health, says that due to limitations in RCTs, which include limited time duration, the relatively small and selective patient populations and the controlled conditions under which they are implemented, there are "uncertainties regarding real life effectiveness, cost effectiveness and health outcomes of a new drug at the time of its assessment for reimbursement". This can influence the ability to establish the real added value of a medicine when a new drug is launched. Ferrario stresses that it is essential that existing data collection systems at national and EU level are further strengthened and integrated in order to demonstrate personalised medicine's added value. She adds that, "harmonising and reducing data requirements between regulators and reimbursement authorities could help to reduce and simplify the regulatory, pricing and reimbursement process, thus enabling earlier access to patients". In conclusion, if value-based pricing is to be achieved, "we need to explore and test new pricing methods which reward the manufacturer for its R&D efforts while ensuring fair prices for healthcare payers," she says. Ansgar Hebborn, head of market access policy for Roche, calls for reimbursement models to be tuned so as to allow access to personalised medicine for "everyone who can benefit". "Personalised medicine can only make a difference to the lives of patients if HTA, pricing, reimbursement and funding pathways for co-dependent pharmaceutical and diagnostic technologies are well aligned."

> *"We need to explore and test new pricing methods which reward the manufacturer for its R&D efforts while ensuring fair prices for healthcare payers"*
> *Alessandra Ferrario*

Cost effectiveness?

A new lung cancer drug which could benefit up to 550 UK lung cancer patients a year has been ruled too expensive for use in Britain's National Health Service (NHS). The drug is a targeted treatment working only on cancer containing a specific abnormal gene. Patients will be unable to access the vital new drug, Crizotinib, which holds many benefits, including fewer side effects, increased ease of use through its availability in pill form, and its outperformance of traditional chemotherapy methods. Despite this, the UK's National Institute for Health and Clinical Excellence (NICE) has ruled that it is not cost effective and would not be recommended for use on the NHS. Dr Jesme Fox, medical director of the Roy Castle Lung Cancer Foundation, said: "We are very disappointed that lung cancer patients in the UK will be denied a new therapy, which is routinely available in other parts of the world. "It is clear that clinically this is a good drug, which would benefit some lung cancer patients. I would urge the drug's manufacturer Pfizer and NICE to urgently come together to discuss the price issue and ensure this is routinely available to all lung cancer patients who would benefit."

Dr Michael Peake, from the UK's National Cancer Intelligence Network, was also critical of the ruling, saying, "As someone who cares for lung cancer patients on a regular basis, I am personally very saddened by this decision. Advanced lung cancer is an aggressive disease with very poor outcomes for many patients. Clinicians recognise the urgent need for personalised medicines which target the specific drivers of an individual patient's tumour."

Due to very few new cancer drugs receiving NICE approval, many patients in the UK are accessing innovative medicines through the government supported Cancer Drugs Fund, but this offers no guarantee of future availability for these treatments and ceases operation entirely in March 2014.

Education, education, education

Educating the next generation of healthcare professionals to navigate an increasingly complex medical landscape is vital

With medicine undergoing a fundamental shift in the way it thinks about patients, treatments and disease, healthcare professionals are in need of up-to-date training and education to help them adapt to these changes.

President of the European oncology nursing society (Eons) Birgitte Grube said that it is vital to "create education for nurses and all healthcare professionals" that matches the new medical environment. For Grube, though, it was important to ensure that the term personalised medicine was properly defined. "Are we only talking about a medical model of the systematic use of genetic or other information to select or optimise an individual preventative or therapeutic care?" Grube stressed that the nursing profession also considered the term to "include all decisions and practices being tailored to individual patients and a more holistic approach". She also highlighted four areas that nurses require more information on if they are to provide professional personalised care: "the functioning of the immune system, knowledge of targeted therapies, the mechanisms of these types of treatments, and adverse effects". "The next generation of nurses, researchers physicians should receive a solid education in these areas." However, Grube insisted that "all levels, including undergraduate and postgraduate" should be educated in personalised medicine techniques, including "how to communicate this knowledge effectively to the public".

Angelo Paradiso, scientific director of the national cancer research centre istituto tumori, said that "knowledge of biologies has greatly improved" and diseases are now "extremely characterised". "We need expertise for new techniques" if the "use of a biomarker is to be optimised", he added. Paradiso was critical of the fact that education activity is mostly being performed in "basic research and not in personalised clinical application of this information". "We need to promote the development of specific professional education," he stressed.

Offering high quality and well-structured post graduate training that is easily accessible is key, according to Carin Smand, managing director of European hematology association (EHA). This is especially important in personalised medicine. Access to this knowledge is crucial and supports further development of personalised medicine in all its aspects. Therefore, said Smand, the EHA structured its education programme based on the European hematology curriculum which is endorsed by 27 European countries. For each item in this curriculum education material is available. In order to make it easily accessible we are currently integrating all our educational tools into one platform.

Romanian EPP deputy Petru Luhan said that the European parliament was investigating "specific tools to transfer knowledge from one country to another, especially in new member states", underlining how important it was that the needs of the personalised medicine sector were "reflected in legislation". "We need to get all the relevant stakeholders at the table," he said. Giovanni Pacini, from Italy's institute of biomedical engineering, said there is a "disappointing situation in Europe with a lot of heterogeneity, no harmonised curricula, and jealousies between different institutions". "It would be good to have a European medical science training programme," he said, stressing that it is vital to start producing professionals with "multidisciplinary research skills". He also said that this would require the formation of "pan-European recognition schemes" for different types of education, facilitating mobility among students.

The commercial director for Science|Business Duane Schulthess stressed that "currently, only 20 per cent of the knowledge clinicians are using is evidence-based". However, he also said the amount of data output would "take a normal clinician 160 hours a week to internalise". "We need to give people the training to filter this correctly and decipher what isn't important," he added.

Professor consultant for the European Science Foundation Kirsten Steinhausen says the key competencies for personalised medicine are interdisciplinarity and interprofessionality

"Personalised medicine has to deal with a high amount of data and information. To implement new methods and techniques the important skills will be to analyse and interpret complex biological, environmental and lifestyle data. This includes competencies for a critical review of information with appropriate methodological knowledge. Education should be interdisciplinary from the earliest stages of professional development.

Communication skills are also important. People who are involved with personalised medicine need to communicate well so that the different disciplines can cooperate efficiently and that the innovative concept of personalised medicine can be explained to citizens, policymakers, regulatory bodies and the media.

Personalised medicine is reflected by a new disease classification system informed and characterised by specific physiological and pathological processes that can be recorded and influenced by each individual. This necessitates more interdisciplinarity in education and training and the curricula. All stakeholders involved – from healthcare professionals and bioscientists, up to patients and citizens – must receive adequate education and training."

Pioneering research

The Innovative Medicines Initiative (IMI) is pioneering an open, collaborative approach to drug research and development in Europe to help drive the development of personalised medicines in diverse disease areas.

The huge burden of chronic and degenerative diseases is rising as Europe's population ages. Tackling complex diseases is scientifically challenging and expensive, and the cost of developing new vaccines, medicines and treatments is soaring.

How can the EU provide priority medicines for all and keep public budgets under control? No government, industry sector or research community can overcome these challenges on its own. Cooperation at EU level between the public and private sectors is the only way forward.

Research is already delivering personalised medicines for diseases such as cancer, and it is becoming increasingly clear that a tailored approach to treatment is needed in several other areas, such as brain disorders and rheumatic disease. Identifying the different diseases that are currently considered to be one disease and developing tests to diagnose them and treatments to tackle them requires a large-scale collaborative research and innovation effort involving all key players in drug development.

The transition from research into practical outcomes for patients is crucial to the development of personalised medicine. Currently, direct and timely implementation of medical treatments is slow and can take up to 15 years, and increasingly empowered patients are demanding a quick and efficient transition from scientific process to available treatment.

With a €2bn budget, the Innovative Medicines Initiative (IMI) is the world's largest public-private partnership (PPP) in life sciences research; the EU contributes €1bn to IMI through the seventh framework programme (FP7); this is matched by in-kind contributions worth at least another €1bn from member companies of the European Federation of Pharmaceutical Industries and Associations (EFPIA).

IMI has been a great success and it is already advancing personalised medicines development by bringing together experts from academia, research centres, the pharmaceutical industry, small and medium-sized enterprises, hospitals, regulators, and patient groups.

On 10 July, the European commission launched a proposal for IMI 2, to be supported jointly by the EU and industry under Horizon 2020. The proposed strategic research agenda for IMI 2 has a strong focus on 'The right prevention and treatment for the right patient at the right time'. IMI 2 will pave the way for breakthrough vaccines, medicines and treatments to tackle Europe's growing health challenges. It will help secure the future international competitiveness of Europe's pharmaceutical industry.

IMI 2's estimated budget stands at €3.45bn, split equally between the EU and the private sector. It is expected to start in January 2014 and end in 2024 and will bring together public sector experts with members of EFPIA as well as partners form other industries and sectors.

Stakeholder perspective

Personalised medicine launches a new era of healthcare that will allow for a more targeted approach at a much earlier stage in treatment. These treatments focus on the individual patient's genetic signature and its dynamic interaction with other health determinants, such as environmental and lifestyle factors.

Science has led to advances that hold great promise for medicines development, including an improved understanding of the genomics of disease, the discovery of biomarkers, the development of new statistical methods, and the invention of dynamic tools for collecting real world data on drug effectiveness and safety. While science and technology have progressed, the basic R&D and regulatory processes remain largely unchanged. As a result, the process of developing new medicines has steadily become more burdensome. Simply stated, it is a matter of the R&D, associated regulatory process, and incentives needing to catch up with the science.

Integrating the new knowledge and science into regulatory pathways requires a departure from the current symptom-based approach to disease and a move towards progressive development and medicines approval models. There is also clearly a need for simplification of the complex and inflexible regulatory procedures within the current framework, and for a review of the supportive incentive system.

Admittedly, such changes aren't simple. Early and ongoing collaboration is required, between various industry sectors and with patients, regulators and payers. Better integration of patient views in research and regulatory decision making are key to this process. With a collaborative effort, the regulatory environment can be updated to match the advances of research paradigms to allow patients earlier access to valuable innovative therapies and prevention agents.

Magda Chlebus is director of science policy at the European Federation of Pharmaceutical Industries and Associations

A call to action

The European Alliance for Personalised Medicine (EAPM) brings together Europe's leading healthcare experts, organisations and institutions, as well as patient advocates. EAPM aims to improve patient care by accelerating the development, delivery and uptake of personalised healthcare.

Personalised medicine can deliver 'the right treatment to the right person at the right time', improving outcomes for patients, reducing side effects and the use of unnecessary and expensive treatments.

EAPM calls on the European commission, MEPs and member states to encourage the introduction of personalised medicine in this legislature and the next by:

1. Ensuring a regulatory environment which allows early patient access to novel and efficacious personalised medicine
2. Increasing research and development for personalised medicine
3. Improving the education and training of healthcare professionals
4. Acknowledging new approaches to reimbursement and public health assessment tools, including HTA assessment, which are required for patient access to personalised medicine and for its value to be recognised
5. Increasing awareness and understanding of personalised medicine

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