EU Options for Improving Access to Medicines

Study for the ENVI Committee

2016
Abstract

This document summarises the presentations and discussions of the workshop on access to medicines, held at the European Parliament in Brussels on Thursday 14 July 2016. The purpose of the workshop was to discuss the latest trends and the current situation as regards the availability of affordable medicines in Europe, to present a range of EU initiatives, and to raise awareness on prices, accessibility, acceptability, affordability and availability of medicines in the EU.

During the first part of the workshop, the current situation on access to medicines was analysed by different stakeholder groups. The second part of the workshop analysed pharmaceutical systems in the EU, covering topics such as intellectual property, pricing and reimbursement systems, and competition enforcement laws.

The third part of the workshop concerned authorisation procedures and systems for pricing and reimbursement. Here, an overview of the role of the EMA, the benefits of health technology assessment and improving affordability were presented. Proposals to improve access to medicines were discussed in the final workshop session.

This workshop and the respective document were prepared by the Policy Department A at the request of the Committee on Environment, Public Health and Food Safety.
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<tr>
<td>AIDS</td>
<td>Acquired Immune Deficiency Syndrome</td>
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<tr>
<td>AIM</td>
<td>International Association of Mutual Benefit Societies</td>
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<tr>
<td>BENELUX</td>
<td>Belgium, the Netherlands, Luxembourg</td>
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<td>CEE</td>
<td>Central and Eastern Europe</td>
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<td>CEO</td>
<td>Chief Executive Officer</td>
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<tr>
<td>DAA</td>
<td>Direct Acting Antiviral</td>
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<td>DG ENVI</td>
<td>Directorate General for the Environment</td>
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<td>DG COMP</td>
<td>Directorate General Competition</td>
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<tr>
<td>DG SANTE</td>
<td>Directorate General for Health and Food Safety</td>
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<tr>
<td>DNDI</td>
<td>Drug for Neglected Diseases Initiative</td>
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<td>EC</td>
<td>European Commission</td>
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<td>ECPM</td>
<td>European Course in Pharmaceutical Medicine</td>
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<td>EFPIA</td>
<td>European Federation of Pharmaceutical Industries and Associations</td>
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<td>EP</td>
<td>European Parliament</td>
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<td>EPF</td>
<td>European Patients’ Forum</td>
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<td>ENVI</td>
<td>Committee on Environment, Public Health and Food and Safety</td>
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<td>EMA</td>
<td>European Medicines Agency</td>
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<tr>
<td>ESIP</td>
<td>European Social Insurance Platform</td>
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<td>EU</td>
<td>European Union</td>
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<td>EUI</td>
<td>European University Institute</td>
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<td>EUnetHTA</td>
<td>European Network for Health Technology Assessment</td>
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<tr>
<td>GDP</td>
<td>Gross Domestic Product</td>
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<td>HELICS</td>
<td>Hospitals in Europe Link for Infection Control through Surveillance</td>
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<td>HCV</td>
<td>Hepatitis C virus</td>
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<td>HIV</td>
<td>Human Immunodeficiency Virus</td>
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<td>HTA</td>
<td>Health Technology Assessment</td>
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<td>IMI</td>
<td>Innovative Medicines Initiative</td>
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<td>Acronym</td>
<td>Full Form</td>
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<tr>
<td>ISPOR</td>
<td>International Society for Pharmacoeconomics and Outcomes Research</td>
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<td>IT</td>
<td>Information Technology</td>
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<tr>
<td>KCE</td>
<td>Belgian Health Care Knowledge Centre</td>
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<td>MDM</td>
<td>Médecins du Monde</td>
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<td>MEA</td>
<td>Managed Entry Agreements</td>
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<td>MEDEV</td>
<td>Medicine Evaluation Committee</td>
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<td>MEP</td>
<td>Member of European Parliament</td>
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<td>MPP</td>
<td>Medicines Patent Pool</td>
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<td>MSF</td>
<td>Médecins Sans Frontières</td>
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<td>NGO</td>
<td>Non-Governmental Organisation</td>
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<td>NIHR</td>
<td>British National Institute for Health Research</td>
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<td>MS</td>
<td>Member State(s)</td>
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<td>PISCE</td>
<td>Platform of Experts on Self-Care</td>
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<td>PRIME</td>
<td>Priority Medicines scheme</td>
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<td>R&amp;D</td>
<td>Research and Development</td>
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<td>RCT</td>
<td>Randomised Controlled Trial</td>
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<td>SEAPA</td>
<td>Health Service of the Principality of Asturias</td>
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<tr>
<td>S&amp;D</td>
<td>Socialists &amp; Democrats</td>
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<tr>
<td>SME</td>
<td>Small and Medium Enterprises</td>
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<td>SPC</td>
<td>Social Protection Committee</td>
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<td>STAMP</td>
<td>Safe and Timely Access to Medicines for Patients</td>
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<td>TRIPS</td>
<td>Trade Related Aspects of Intellectual Property Rights</td>
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<td>UAEM</td>
<td>Universities Allied for Essential Medicines</td>
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<td>UK</td>
<td>United Kingdom</td>
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<td>UN</td>
<td>United Nations</td>
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<td>US</td>
<td>United States</td>
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<td>WHO</td>
<td>World Health Organisation</td>
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<td>WTO</td>
<td>World Trade Organisation</td>
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EXECUTIVE SUMMARY

On 14 July 2016, the Committee on Environment, Public Health and Food and Safety (ENVI) of the European Parliament held the workshop, ‘EU options for improving access to medicines’. The workshop was hosted by Ms Soledad CABEZÓN (MEP, S&D), who opened with an explanation that the outcomes of the workshop will feed directly into an own-initiative report that she is currently preparing and that will be discussed by the ENVI Committee on 29 September 2016. Mr José Inácio FARIA, shadow rapporteur of the workshop, added that access to medicines is a generalised human need, and that the workshop would focus on identifying ways to improve the situation for citizens in Europe.

The first session of the workshop was opened by Mr Paul VAN HOOF of the European Federation of Pharmaceutical Industries and Associations (EFPIA). He noted that innovation has contributed to better management of diseases and an increased life expectancy for the overall population in Europe. Mr van Hoof highlighted the importance of engaging with healthcare stakeholders, particularly with respect to innovative pricing models.

Mr Adrian VAN DEN HOVEN, Director General of Medicines for Europe, spoke next, focusing on the stimulation of competition in pharmaceutical markets. He provided a brief overview of the current healthcare system and medicines in Europe, and outlined the reasons which, he believes, should place competition at the top of the pharmaceutical agenda. He also raised the issue of medicine shortages driven by extreme pricing policies, and put forward some suggestions for addressing this situation.

Mr François FILLE, European Advocacy Coordinator at Médecins du Monde, presented the NGO perspective, referring to the prohibitive prices of some of the current medicines, which, he stated, endanger public health. He suggested that patent opposition and compulsory licensing are appropriate tools to address this issue. He concluded by highlighting the importance of ensuring transparency in the research and development costs in health budgets.

Ms Kaisa IMMONEN, Director of Policy of the European Patients’ Forum (EPF), delivered a statement on the role of patients’ associations in access to medicines. She reiterated patients’ fundamental right to access to medicines, emphasising that treatment should be based upon needs rather than means. Universal access to medicines is a political choice, she said, and should be at the top of the EU agenda.

Dr Ellen ’t HOEN, from the Medicines Law & Policy and Global Health Unit of the University Medical Centre (Groningen), opened the second panel with a presentation on the intellectual property law system applicable to medicine. She described the struggle of Member States in providing for high-priced medicines in their health budgets, and outlined some strategies for coping with this reality.

Prof. Dr Lieven ANNEMANS, from the University of Ghent, gave an overview of European systems for prices and reimbursement of medicines. He stressed that access to medical care is provided for by the Universal Declaration of Human Rights and that such healthcare involves quality, solidarity and sustainability. He concluded by highlighting the need for increased investment, more innovation, and greater equality in order to improve access to medicines.

Mr Dirk VAN ERPS, Head of the Antitrust Unit of the European Commission, spoke about ‘Competition enforcement for pharmaceutical products’. He outlined the competition process and presented the outcomes of the 2009 Report on Pharma Sector Inquiry. He outlined the DG COMP focus on pay-for-delay agreements, and compared assessment ‘by object’ and ‘by effect’.
Ms Enrica ALTERI, European Medicines Agency, opened the third panel, ‘Authorisation procedures and systems for pricing and reimbursement’. She described the process by which medicines are designed and approved before being made available to patients. She referred to the EMA-HTA Parallel Scientific Advice and described the PRIority MEdicines (PRIME) scheme.

Dr Raf MERTENS, from the Belgian Health Care Knowledge Centre (KCE), focused on Health Technology Assessment (HTA). He referred to the comparative effectiveness of external validity and to adaptive pathways, and stressed the need for non-commercial trials. He referred to the relationship between cost and effectiveness and showed how high-priced new medicines have been handled to-date within the EU.

Dr Fernando LAMATA, expert in healthcare policies, talked about the authorisation, pricing, reimbursement and purchase processes. He referred to the existence of funding and monopoly markets in the EU pharmaceutical industry, and explained the strategies by which abuse of such a monopoly can be averted. He concluded by putting forward proposals for the control of prices and subsequent increase of affordability.

The fourth and final panel of the workshop opened with a presentation by Mr Aquilino ALONSO MIRANDA, Health Minister of the Andalusian Government. Mr Alonso Miranda described the innovative methods used by the Andalusian Public Health administration to improve affordability, such as issuing prescriptions by active ingredient and the use of e-prescriptions.

Mr Pierre MEULIEN, Executive Director of the Innovative Medicines Initiative (IMI), spoke next, detailing the role of IMI in research, development and innovation in medicines. Mr Meulien explained the relevance of the IMI to the attempt to find more affordable and readily available medicines. He described three examples of IMI projects which aim to tackle concrete challenges in drug development.

Mr Maurice GALLA, Senior Policy Officer and EU Presidency Coordinator of the Dutch Ministry of Health, Welfare and Sports, spoke next. After recapping the priorities of the recent EU Presidency by the Netherlands, Mr Galla referred to the informal meeting of Health Ministers and to the main conclusions of the Council, drawing particular attention to the actions for Member States and the Commission.

The final speaker of the afternoon was Mr Andrzej RYS, Director of the Unit Health Systems, Medical products and innovation of the Directorate-General SANTE at the European Commission. Mr Rys opened his presentation, ‘Improving access to medicines in the EU’, with a diagnosis of the challenges in the sector. He also referred to the debate on access to medicines at European level.

Closing remarks came from Mr José Inácio FARIA, MEP, shadow rapporteur, who highlighted the importance of adopting high standards of transparency, and Ms Soledad CABEZÓN, MEP and workshop Chair, who emphasised the importance of developing more sound regulations and improving the system of access to medicine through a patient-centred focus.
1. LEGAL AND POLICY BACKGROUND

According to the World Health Organisation (WHO), access to essential medicines is part of the right to health\(^1\). This access to health treatment, however, is heavily dependent on the availability of affordable medicines. Recent findings show striking differences in the sales and availability of innovative medicines between different EU countries\(^2\). This can be the result of several factors, including pricing and reimbursement systems, logistical supply and storage problems, low drug quality, inadequate production and inappropriate use, as well as the often rigid patenting rules.

The economic crisis exacerbated the problem of unequal access to pharmaceuticals and medicinal products in the EU\(^3\), with the growth in healthcare spending collapsing in 2010 as a result of the crisis, and countries such as Greece experiencing serious shortages of medicines\(^4\). A recent WHO report examined the current evidence base of sustainable access to new medicines across Europe, highlighting the need to improve efficiency in spending while maintaining an appropriate balance between access, innovation and cost-effectiveness\(^5\). The report also stressed the need for improved cooperation between countries, together with the suggestion that dialogue with stakeholders should be strengthened to determine what constitutes a fair reward for industry innovation while still preserving access to medicines for patients.

Decisions on pricing of medicines are a national competence, creating a variety of national pricing and reimbursement schemes across the EU. Such different systems may lead to disparities in pricing, time-to-market delays and access inequalities. There is a European dimension to availability and affordability of medicines, as national decisions must comply with Directive 89/105/EEC\(^6\), which requires pricing and reimbursement decisions to be made in a timely and transparent manner. Member States are, however, entitled to make political choices on the medicines they reimburse, in line with the provisions of the Transparency Directive\(^7\). They may make decisions based on the effectiveness and value for money of innovative medicines, as well as their own budgetary constraints.

The importance of ensuring access to medicines for all citizens in Europe has been addressed by a range of EU initiatives. In 2008, for example, the European Commission adopted a Communication aiming to, *inter alia*, launch reflections on ways to improve market access\(^8\), as well as three legislative proposals with the objective of ensuring that European citizens

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can benefit from safe, innovative and accessible medicines. The legislative proposals ensure that EU citizens have access to reliable information on medicines (COM(2008) 663 final)\(^9\), strengthen the EU’s system for safety monitoring of medicines (pharmacovigilance) (COM(2008) 665 final)\(^10\) and protect EU citizens from threats posed by fake medicines (COM(2008) 668 final)\(^11\).

The Commission also took steps to facilitate discussion on ethics and transparency as well as on non-regularity conditions for better access to medicines after receiving a marketing authorisation. Between 2010 and 2013, it set up the ‘Process on Corporate Responsibility in the Field of Pharmaceuticals’\(^12\). As part of the process, DG GROW initiated a ‘Platform on Access to Medicines in Europe’, bringing together EU countries and other stakeholders to find common, non-regulatory approaches to timely and equitable access to medicines in Europe after their marketing authorisation\(^13\). The Platform implemented several projects (e.g. priority medicines, managed entry agreements, small markets, orphan drugs and biosimilars), to which all of its members contributed.

Another initiative put in place by the European Commission to tackle the issue of access to medicines is the Expert Group on Safe and Timely Access to Medicines for Patients (STAMP). This group provides advice and expertise to the Commission services in relation to the implementation of EU Pharmaceutical legislation and relevant programmes and policies\(^14\). The STAMP exchanges views and information about the experiences of Member States, examines national initiatives and identifies more effective ways to use the existing EU regulatory tools to improve safe and timely access and availability of medicines for patients.

A joint procurement agreement was adopted by the Commission in 2014, at the request of the Council for communicable diseases. This enabled Member States to purchase medicines together in order to help to secure access to medicines at better conditions and prices\(^15\). During that same year, a Strategy for EU cooperation on Health Technology Assessments (HTAs) was adopted by the Commission, to reduce fragmentation in the evaluation of technologies and to foster the exchange of information among European HTAs, ultimately speeding up access to innovative treatments\(^16\).

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Another EU initiative is the ‘Network of Competent Authorities on Pricing and Reimbursement’, established during the Slovenian Presidency in 2008\(^{17}\). The Network offers Member States’ authorities the opportunity to identify, share and discuss information, expertise and best practices/policies on high level issues relating to pricing and reimbursement of pharmaceuticals. The Network’s discussion take place in different political and technical fora, such as the Council Working Party on Public Health at Senior Level\(^{18}\), the European Network for Health Technology Assessment (EUnetHTA)\(^{19}\), and the STAMP. The importance of cooperation between authorities was also highlighted by the Commission in its Staff Working Document of June 2014: ‘Pharmaceutical Industry: A Strategic Sector for the European Economy’\(^{20}\).

The topic of access to medicines has been debated by the European Parliament in plenary in 2014 and 2015\(^{21}\). The last session focused on the extremely high prices for certain life-saving medicines (e.g. Sofosbuvir, a treatment against hepatitis C, and certain cancer drugs), and raised questions about the rationale behind prices, price negotiations and evaluation of the benefits of the new products. The origin of the chain, and the conditions for the financing of medicines were also discussed\(^{22}\). At the beginning of June 2016, the Group of the Progressive Alliance of Socialists & Democrats (S&D) of the European Parliament adopted recommendations aiming to overcome obstacles and reduce inequalities in access to medicines in the EU\(^{23}\). Such issues have become particularly acute in the wake of the economic crisis, with additional pressure stemming from increases in the costs of medicines and the overall ageing of the European population.

Despite the discussions and initiatives at EU level, the regulatory landscape in the EU remains complex, and challenges still exist. Ensuring equal access to medicines across the EU can be fostered by exchanging knowledge and experiences between national authorities on the diversity of national pricing and reimbursement schemes, as well as initiating dialogue with wider stakeholder groups. Further research is needed on methods by which market access for medicines may be sped up. Possible areas of benefit include further exploring the use of HTAs in striking a balance between containing pharmaceutical expenditure and ensuring a fair reward for valuable innovation and access to medicines, testing and exploring different pricing and financing models, and optimising data gathering.


PROCEEDINGS OF THE WORKSHOP

1.1. Introduction

1.1.1. Welcome and opening

**MEP Ms Soledad CABEZÓN, Chair, ENVI Health Working Group**

Ms CABEZÓN thanked participants for taking the time to attend. She stressed the timeliness of the event, explaining that its outcomes will be used for an own-initiative report on EU options for improving access to medicines, due to be published later this year. Ms Cabezón drew attention to the different facets of the issue of access to medicines, and concluded by stating that industry experts and patient associations would all have the opportunity to express their point of view.

**MEP Mr José Inácio FARIA, Shadow rapporteur**

Mr FARIA added that the workshop was attended by some 120 individuals from a variety of backgrounds, including patients’ associations, experts and citizens. He pointed to the universal nature of concerns about access to medicine, given that good health is a prerequisite for almost everything in life. Citizens seek medicines that are easily available and affordable, in addition to being safe effective and of high quality. A multitude of factors influence this availability and affordability, such as pharmaceutical research, the supply system, company strategies, pricing and reimbursement. Mr Faria insisted that these factors must be analysed in order to find adequate ways of overcoming obstacles and reducing inequalities in access, availability and pricing. Mr Faria concluded that he was confident that this workshop would shed further light on these issues and he welcomed input from various points of view.

1.2. Part I: Access to Medicines from Different Points of View - Analysis of the Current Situation

1.2.1. Pharmaceutical industry of medicines produced in Europe

**Mr Paul VAN HOOF, European Federation of Pharmaceutical Industries and Associations (EFPIA)**

Mr VAN HOOF started by stressing that the primary commitments of the European Federation of Pharmaceutical Industries and Associations (EFPIA) are to reduce inequalities in terms of healthcare, to accelerate patients’ access to innovative medicines and to improve patient safety and quality of life. Medicines can, he said, contribute to this aim in a significant way, such as the increases to life expectancy in the last decade. He also pointed to the considerable progress made towards finding new solutions to combat diseases, such as cell and gene therapy, and bioelectronics. As a result of these innovations, the percentage of death caused by diseases like HIV and cancer has fallen by 20%, and more patients can be cured of diseases such as Hepatitis C.

Mr van Hoof went on to explain that, to-date, EFPIA has developed 7,000 new medicines, of which more than 200 are medicines for blood cancer. He stated his belief that this new wave of medical innovation will play a key role in addressing the challenges faced by patients and healthcare systems. He acknowledged, however, that there are concerns about affordability and access to medicines, as well as pressure on the budgets of governments and healthcare
systems. These concerns are shared by EFPIA members, who work to enhance access to medicines in Europe.

In order to address this issue, EFPIA is concluding pharmaceutical stability agreements at national level. These agreements are undertaken between the biopharmaceutical industry and governments, and aim to balance access to medicines for patients with a more predictable and manageable pharmaceutical budget, while maintaining a good return for pharmaceutical industries. In those countries where such agreements have been put in place, he said, they seem to provide a good base for managing both access and budget concerns. Many EFPIA members are also establishing managed entry agreements for new medicines. These agreements balance access for patients with uncertainty of product affordability in the longer term.

Despite these efforts, Mr van Hoof believes that further steps are needed. Socioeconomic gaps, as well as gaps in terms of GDP and affordability, are widening in the EU, increasing inequalities in access to medicines. These gaps should be addressed by enabling governments to implement a system of differential pricing. At the same time, those industries that are making innovation possible should be rewarded. He pointed to the need to build sustainable healthcare systems based on new pricing models and value based contracts. In order to make this happen, EFPIA advises a collaborative approach, with all stakeholders participating in order to achieve a sustainable system.

Mr van Hoof placed particular emphasis on the need for some urgent solutions, such as developing new antibiotics and creating incentives for innovation. Intellectual property rights remain necessary to protect and reward innovation, given the high-risk nature of pharmaceutical research and development (R&D), where several failures may precede an innovative product. Only one in ten medicines at the clinical trials stage of development will ever reach patients. Pharmaceutical industry therefore needs continued return on its investment, and Mr van Hoof stressed the importance of balancing the need to have medicines accessible for all while protecting innovation whose value is felt by all of society. Given its belief in the importance of intellectual property rights, EFPIA welcome the Council Conclusions in this regard24.

Mr van Hoof underlined the importance of patient outcomes and healthcare efficiencies in creating sustainable healthcare systems, pointing once more to the importance of collaboration and dialogue among stakeholders. He concluded by stating that the focus should remain on improving patients’ lives by providing accessible medicines and sustainable healthcare systems, while still rewarding innovative industries.

1.2.2. Medicines for Europe, the voice of the generic, biosimilar and added-value medicines in Europe

Mr Adrian VAN DEN HOVEN, Director General, Medicines for Europe

Mr VAN DEN HOVEN began by explaining that the association he represents has recently been rebranded to ‘Medicines for Europe’. It represents three sectors of manufacturing: generic medicines, biosimilar or biological medicines and value-added medicines. He underlined that the activities of Medicines for Europe place a strong emphasis on improving access to medicines for patients in Europe.

Mr van den Hoven described some of the key healthcare challenges faced by Europe. He agreed with Mr van Hoof’s affirmation that many more patients need treatment these days, as evidenced by 2% increase per year in the consumption of medicines in Europe. Additional pressures are brought to bear from increasing treatment costs and the high costs of new drugs for small patient populations, while austerity measures, such as short-term cost cutting, undermine sustainable healthcare systems and long-term efficiency models.

Despite the very good research pipeline that has been put in place in the field of medicines, Mr van den Hoven explained, generic competition is declining or stagnating, particularly in countries such as Spain, France, Portugal and Romania. He attributed this to weak policies that fail to sustain competition, as well as the shortages of essential drugs as a result of extreme pricing policies. Mr van den Hoven then analysed the development of the medicines market in 2014, highlighting the significant increase in development stemming from a very good research pipeline that finally reached the market. This development, however, had a high cost impact, as it impacted on only a small part of the population. He referred to this as the ‘speciality space’, relating to diseases such as hepatitis, cancer and autoimmune diseases. More generic competition is needed if the cost of such products is to be reduced.

Medicines for Europe, he explained, sees generic competition and prevention as drivers of efficiency in healthcare, which is why they believe that competition should be at the top of the pharmaceutical agenda. This point was also raised by the EU Health Council Conclusions, which called for more competition in the pharmaceutical markets for generic and biosimilar medicines. This is significant, given that generic medicines represent 56% of the medicines consumed by European patients and given the potential of biosimilar medicines to bring significant savings.

Mr van den Hoven then gave specific examples of measures that Medicines for Europe proposes to improve access to medicines in Europe. Member States should, for example, stimulate competition through uptake measures for generic and biosimilar medicines. They should analyse specialty markets closely to determine how best to generate competition in that space. Generic, biosimilar and value-added developments should be included in 'horizon scanning' to balance pharmaceutical markets and, finally, procurement models should be used to avoid shortages of essential medicines.

Mr van den Hoven concluded by stating that Medicines for Europe supports a multi-stakeholder approach for specialty competitions (i.e. competition from biosimilar medicines, complex generics, value-added medicines) in order to build trust and stimulate market competition in an open and transparent way.

1.2.3. NGOs and access to medicines

Mr François FILLE, European Advocacy Coordinator, Médecins du Monde

Mr FRANÇOIS FILLE started by stating that access to medicines is a major global problem affecting 185 million people, five million of whom are European. EU countries chiefly face difficulties in ensuring access to medicines due to extreme pricing. This is particularly the case for medication to treat the hepatitis C virus (HCV), which led Médecins du Monde (MDM) to increase its involvement in HCV treatment and diagnosis. Their increased involvement coincided with the arrival of the new generation treatment ‘Direct Acting Antiviral’ (DAA), which reduces mortality in 90% of cases and makes the eradication of HCV a real possibility.

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Mr Fille explained, however, that the prices of DAAs are exorbitant, and have been set arbitrarily and without a connection to R&D.

These high prices for medication have created an enormous financial burden for EU countries already struggling with budgetary restrictions. Further financial weight is imposed by the lack of offer, or limited offer, where access is based on a ‘cost-effectiveness evaluation’. Mr Fille gave the example of France, where the provision of sofosbuvir for all patients at the actual price would be equivalent to the annual budget of all Parisian public hospitals or the French public health insurance deficit. The current price leads to treatment rationing and endangers the sustainability of the public health system, which MDM considers unethical and ineffective from a public health perspective.

MDM believes that the solution to this sofosbuvir problem may first lie in the patent issue. With the patent of sofosbuvir in place until 2029, the monopoly position encourages high pricing by the patent holder. Following positive outcomes in India and Egypt, MDM has filed a challenge to the patent at the European Patent Office, and a decision is expected to be reached late 2016.

A second solution to improve access to medicines may be found among several legal tools, such as compulsory licences. Mr Fille explained that MDM considers compulsory licence an essential safeguard to overcome misuse of intellectual property rights by patent holders. This is in line with an WHO opinion paper, which states that compulsory licences remain one of the most effective ways of ensuring access to drugs while preventing licence abuse. MDM has therefore requested, together with citizens and the French authorities, that a compulsory licence be used to decrease of the price of sofosbuvir. This request was refused, however, because, despite being designed to overcome overpricing policies, compulsory licence is still considered too radical.

Mr Fille concluded with a reminder that the US was one of the first countries to threaten a pharmaceutical corporation with compulsory licence during the anthrax threat, which resulted in savings of USD 97 billion. Drug prices should thus be viewed not only as an aspect of innovation but also as a public health matter. He recommended initiating a public debate on drug pricing, more transparency on R&D costs from pharmaceutical companies and a dramatic reduction in prices of medicines, specifically sofosbuvir.

1.2.4. Patient associations and access to medicines

Ms Kaisa IMMONEN, Director of Policy, European Patients’ Forum (EPF)

Ms IMMONEN opened by stating that the EPF’s position on EU access to medicines rests on a fundamental principle of equitable access to treatment based on needs rather than means. While the EPF recognises the economic arguments for investing in health, it points also to the ethical foundation that demands exploration of the long-term sustainability of health systems.

Timely access to accurate diagnosis and appropriate treatment improves health and quality of life. It can also save costs for health and social systems by avoiding unnecessary worsening of health conditions. Ms Immonen recognised, however, that equitable access is not a reality across the EU, with considerable differences between Member States in access to medicines, for example in the treatment of multiple sclerosis.

In order to ensure that the patient perspective is heard, EPF recently published a statement on the patient community’s view of the key points for action on access to medicines and ensuring equality in access to treatments. Ms Immonen pointed to the key message from this paper, which stats that ensuring access requires a joint effort between the pharmaceutical industry, Member States and other stakeholders. Industry should price
medicines in a responsible way, taking into account, for example, a country’s capacity to pay, the impact on budgets and the kind of public funding invested in drug development. The pharmaceutical industry, Ms Immonen stated, needs to do better in ensuring transparency and ethical conduct in order to ‘walk the talk’ as a responsible health stakeholder. Member States need to take political action to ensure universal access and invest in valuable innovation, because patients’ lives cannot be measured only in monetary terms.

Ms Immonen explained that the EPF’s paper calls for a coherent and structured framework for fair access at European level for medicines, particularly innovative medicines. The EPF believes that a set of common principles and mechanisms would encourage innovation while also ensuring sustained investment in medicines. This should include exploring the best use of existing tools to increase access and reduce uncertainty when new medicines are launched. The EPF is aware that such discussions demand a certain political will and strong leadership. She highlighted the recent collaborations between the BENELUX countries and Austria as an important milestone.

This process of collaboration should be done in a more systematic way, and should include patient involvement. Ms Immonen stated that, despite being directly affected by decisions taken by politicians and authorities, patients remain largely uninvolved in policy making. The patient perspective provides a unique insight and must influence the medical needs prioritised in R&D. The EPF recommends that patients are involved in all stages of the process, from the setting of research priorities and early dialogue to regulatory and health technology assessment, including pricing and reimbursement decisions and post-marketing data collection. The EPF hopes that the European Parliament (EP) report will recognise the centrality of the patient’s role.

Ms Immonen concluded that there is a need for a changed mind set and a new collaborative, non-confrontational culture. Dialogue with patients must start early and continue throughout the life cycle of R&D, and the fundamental values of universality, equity and solidarity must underpin all decisions. The EPF believes that a political choice can be made to ensure universal access to medicines, including innovative medicines, for those who need them.

All citizens in Europe face common problems and, together, common solutions must be found that ensure sustainability without undermining access or investment in medicines research. While some voices may, in the current political context, question the entire idea of the EU, this should not divert attention away from health policy. Citizens value health very highly and there must be a level of commitment from policy makers to act at a European level to improve health policies.

1.3. Part II: Functioning analysis of pharmaceutical systems in the EU

MEP Mr José Inácio FARIA, Shadow rapporteur

Mr José Inácio FARIA opened the second panel by reiterating the importance of innovation in the pharmaceutical sector, innovations which have enabled patients to benefit from treatments that were unimaginable a few decades ago. He added that the lack of affordable treatment for many diseases requires continuous innovative efforts, both from the development companies and other stakeholders, such as universities, if new medicines are to continue to be developed.

Intellectual property rights are, Mr Faria stated, a key element in the promotion of innovation. While the protection of intellectual property rights is important for all economic sectors, it is particularly so for the pharmaceutical sector, given the need to address current and emergency health problems and the long life cycle of products. The pharmaceutical sector in the EU relies significantly on intellectual property rights to protect innovation. These
protections operate during limited periods determined by patent law, thereby providing incentives to development companies to pursue continuous innovation, something which is also in the best interests of patients.

1.3.1. Intellectual property law system applicable to medicine

Ms Ellen ’t HOEN, Medicines Law & Policy and Global Health Unit, University Medical Centre, University of Groningen

Ms ’t HOEN started by stating that the function of intellectual property is to encourage investors to invest time and money in R&D by providing exclusive rights for a limited period of time in exchange for an early public disclosure of the invention. The basic thinking behind the current patent system is that if investors are allowed a certain period within which to recover their investment in new treatments, this stimulates investment and affects the pricing of new medicines.

In Europe, as well as in a number of other countries around the world, there is a growing variety of patent and non-patent based market exclusivities, such as the supplementary protection certificate. Other exclusivities are granted through the EMA or national agencies. The remedies that exist to deal with patent barriers, such as compulsory licences, will not apply to these types of exclusivities. Ms ’t Hoen clarified that the market exclusivity system is regarded as a social policy tool, set in place to provide benefits for society, not corporations. She acknowledged, however, that these tools come with a significant cost for society that must be considered, as indicated by the Council Conclusions.

With respect to pharmaceutical innovation, Ms ’t Hoen said, it is not all good news in Europe. Over the last fourteen years, she argued, the vast majority of the new medicines which entered the European market did not offer expanded therapeutic opportunities but simply created a burden on EU citizens. Ms ’t Hoen then referred to an issue of European and global importance, the effects of the orphan drug regulations. These drugs, developed to treat rare medical conditions, are top-selling medicines with global sales of several billions each year. Ms ’t Hoen questioned whether such drugs merit additional exclusivity for the respective corporations.

Next, Ms ’t Hoen showed the results of providing patent holders with strong rights in the determination of prices. Research on target prices shows differences between the US and the EU, as well as differences within the EU. She showed evidence of the reach of the policy space that governments have when taking decisions on pricing, arguing that while commercial businesses can legitimately seek profit, governments should not take the same approach.

The way in which prices of medicines are set is interesting, Ms ’t Hoen said. Pricing discounts demand greater collaboration, government intervention and more dialogue than is currently in place. A key question, both for Europe and globally, is whether or not the benefits generated by the current incentive system of pharmaceutical development outweighs the costs.

Ms ’t Hoen raised the high level panel established by the UN Secretary-General in early 2016, aiming to look at access to medicines and to seek rebalancing obligations to protect both human rights and intellectual property protection. She added that the recent Council

27 For further details, see http://www.unsgaccessmeds.org/.
Conclusions offer important opportunities, such as its request that the European Commission conducts an analysis of the impact of the incentive mechanisms in EU legislative instruments on pricing and on the development of new medicines. According to Ms ‘t Hoen, this offers opportunities to increase cost transparency and explore alternative incentive mechanisms.

Increased collaboration aimed at lowering prices for important medicines is essential and the EU, as an important funder of R&D, should make sure that the return on investment provides a benefit for the public. Now, more than ever, she concluded, the EU must respond to EU citizens’ concerns.

1.3.2. European systems for pricing and reimbursement of medicines

Prof. Dr Lieven ANNEMANS, Health Economics Unit, Department of Public Health, University of Ghent

Dr Lieven ANNEMANS opened with Article 25 of the Universal Declaration of Human Rights, declaring that its content is not achieved today in Europe. The current European Commissioner for Health also recognises that something must be done, he said, as he has indicated the need to invest in health policy, believing that such investments will have positive effects on the economy as a whole. When investing in health policy, three principles must be respected. Firstly, ‘quality’: patients are willing to pay more for medicines with therapeutic benefits. Secondly, ‘solidarity’: people with the same healthcare needs deserve the same quality of treatment. Finally, ‘sustainability’: if financial resources are not spent wisely, the sustainability of future healthcare systems cannot be assured.

In line with the principle of quality, Dr Annemans recognised that investments should only be made in those innovative medicines that clearly offer an added-value to patients or society and which come at an acceptable cost. Feedback from citizens should be considered when defining thresholds and financial limits for access to such medicines. Dr Annemans referred to Health Technology Assessment (HTA), which determines the efficacy of the drug, its cost-effectiveness, social and ethical impact and best practice.

Dr Annemans presented the Belgian approach for medicines, whose laws have four criteria for the decision on price and reimbursement of a new drug: added-therapeutic value; therapeutic needs; impact on the budget; and cost-effectiveness. All four criteria must be assessed in decision-making.

Unequal access to medicines stems from each Member State having its own system for pricing, reimbursement and different social values. During the Belgian Presidency of the EU, the government published a study suggesting that there are three main steps to focus on in order to unify this process. The first is market authorisation (done by the EMA), the second HTA (by competent bodies) and the third price/reimbursement (decided by each Member State).

In its assessment of innovative medicines, Dr Annemans showed that the EMA uses the criteria of efficacy, safety and so-called ‘relative efficacy’. These criteria are again assessed by HTA and competent bodies within the Member States during the next step of the process. This duplication of work depletes financial resources, and Dr Annemans therefore suggested that an integrated approach should be developed, at least for clinical value. Action is needed, he said, while still respecting the subsidiarity principle and the competences of individual Member States. He also endorsed the idea of a common European definition of ‘added therapeutic value’ for different diseases.

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Dr Annemans concluded with a plea for further investments in health, for a joint assessment of relative effectiveness and added value, and for the application of common principles. These common principles should extend to the assessment of cost-effectiveness and budget impacts, but should also help to address uncertainty of the value of new medicines. If society is unjust, then healthcare will also be unjust, he said, and there is still much to do to reduce inequalities in Europe.

1.3.3. Competition enforcement for pharmaceutical products

**MEP Mr José Inácio FARIA, Shadow rapporteur**

Mr FARIA briefly introduced the presentation of Mr Dirk VAN ERPS, referencing the 2009 European Commission ‘sector-inquiry’ into the pharmaceutical sector. The aim of this inquiry was to examine the reasons for delays to market entry of generic medicines, as well as the reducing numbers of new medicines entering the market.

The inquiry found that the pharmaceutical industry is undergoing significant changes and that the blockbuster medicines (i.e. the medicines with huge annual turnover which account for a substantial part of sales and profits of the large development companies) have lost patent protection in recent years, with more due to do so in the coming years. Despite increasing investment in R&D, development companies are struggling to fill their product pipelines and the number of new medicines reaching the market is decreasing. This, together with other factors, Mr Faria said, makes development companies increasingly dependent on the revenues of their existing bestselling products and, inevitably, they wish to maintain these for as long as possible. This has led to illegal practices, such as paying generic companies to delay the launch of generic medicines. Mr Dirk van Erps’ presentation, he said, would provide additional information on this issue.

**Mr Dirk VAN ERPS, Head of Unit Antitrust: Pharma and Health services, DG COMP**

Mr VAN ERPS opened with a reference to the summary of the 2009 EC sector inquiry carried out by DG COMP. He clarified that DG COMP is neither a price regulator nor a patent legislator, but instead a kind of ‘policeman’ with a role in the access to medicines dialogue. DG COMP believes that, in the long term, if competition can be maintained and companies do not set up restrictive agreements, abuse their dominant position, or merge to create a monopoly, Europe will have more innovation, better quality products and lower prices for medicines. He explained that, at DG COMP, competition is analysed from the technical point of view. In case of a merger, for example, if there is excessive overlap and insufficient research pulse, DG COMP may intervene.

Mr van Erps explained that during the patent and market exclusivity life, whenever an exclusive right or patent is granted to a company, nobody else can put the same product on the market. In these cases, DG COMP seeks to verify that a patent is not being abused through collaborative arrangements on supply or pricing. Since its sector enquiry, DG COMP has focused on ending market exclusivity and allowing generic competition. Mr van Erps explained that generic competition is positive, as it provides incentives for innovation and costs savings.

Mr van Erps outlined the DG COMP focus on pay-for-delay agreements, i.e. agreements through which a developer pays a potential competitor to stay out of the market for a particular product. Before generic medicine enters the market, all profits go to the innovator.

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However, once the generic is introduced, prices go down and profits decrease for the original developer. Two recent cases, still under appeal, where DG COMP found that agreements have caused consumer harm by delaying generic entry and maintaining unnecessarily high prices, are Lundbeck and Servier. To avoid more of such cases, DG COMP has been requesting companies to submit, on a yearly basis, any patent settlement agreements made amongst themselves. The results of these inquiries are published by DG COMP in reports, and only few problematic agreements have so far been identified.

Some investigations are still ongoing, namely in the field of biosimilar medicines. A recent merger decision clearly showed that the model used to regulate generic competition will not be effective for biosimilar medicines, and that new solutions must be developed. Access pricing, Mr van Erps said, receives the most attention in this regard. Finally, he noted the work done by the Commissioner for Competition in defence of innovation, stating that interventions on price to promote investment can have short-term gains but may have the effect of reducing innovation.

1.3.4. Questions & Answers

Ms AUKEN, MEP, (Group of the Greens/European Free Alliance), raised two questions. The first question was addressed to Dr Annemans and concerned the harmonisation of patent regulations to allow for greater transparency and prevent conflict of interest. The second question, addressed to Mr van Erps, referred to the ‘Lundbeck case’. Ms Auken stated that many similar cases on competition issues are still pending and that, to her knowledge, there are more than one big cases on ‘dirty agreements’.

Mr van Erps responded that there is one ongoing investigation in pay-for-delay. Annual monitoring is also carried out, with about 100 companies submitting their patent settlement agreements. No new ‘dirty cases’ - where big sums of money were paid to generics to remain out of the market - have been discovered during these activities.

Dr Annemans replied that the answer to Ms Auken’s question largely depends on the approach taken to pricing, which can be either the cost plus approach or the value-based approach. The cost plus approach is based on the initial cost of R&D, after which the company is given a mark-up on top of this initial cost to obtain the final price. This approach may inadvertently give incentives to the wrong behaviour, allowing companies to spend as much as they want on R&D without any assurance that this makes R&D more efficient. This system also allows a company that has had more failures to justify the high price of a drug that was finally successful. He stated that value-based pricing is a more reasonable approach, as it stimulates real breakthroughs. However, it is important to clearly communicate the limits in advance, to avoid the development of medicines that are too expensive. It is essential to control profitability and stakeholder payments in order to ensure that the amounts remain within acceptable limits.

Mr van Erps added that the amounts spent in marketing can also be subjected to controls to avoid conflicts of interest. He stated his belief that this is not a problem as long as there is transparency and patient interests are protected.

Ms ’t HOEN stressed that meetings like this one should start with declarations of conflicts of interest. She noted the importance of Mr van Erps point on transparency, given the presence of policy dialogue but no hard facts. There is a rise in demand, she said, for governments and the EU to become more proactive and work together to deliver for citizens and not just commercial entities. The tendency to let governments negotiate medicine prices in secret will no longer work and democratic governments must be accountable to their citizens. Negotiating with a company that has a monopoly in the market is complicated, and increasing collaboration can only improve the measures available to governments in dealing with such
monopolies. Examining the European regulations that hinder governments will clear the way to improve access to medicines. While there is much to be done, greater transparency will allow for a more informed dialogue.

A final question was asked of Mr van Erps by Ms Cabezón, on the ways in which the abuse of the pharmaceutical industry regarding their dominant market position and ability to set excessive prices for medicines can be controlled.

Mr van Erps explained that, in 50 years of competition enforcement, no case focused only on excessive pricing, as all of the cases were also linked to other types of abuse. Where there is real innovation, he said, other factors also come into play. He stressed that innovation must be rewarded as a means of promoting better outcomes, such as further innovation and lower prices. Mr van Erps again pointed out that DG COMP is not a price regulator, that while they can determine if a price is excessive, they cannot establish what the value should be. The market is developing and Member States are cooperating more than ever before, and he advised caution before attacking the system with excessive pricing. He gave some examples of cases deemed unacceptable by DG COMP, such as one developer who sold a generic portfolio to a competitor which then increased the price without any innovation.

1.4. Part III: Authorisation procedures and systems for pricing and reimbursement

1.4.1. Role of EMA in authorising and supporting access to new medicines

Dr Enrica ALTERI, Head of Human Medicines Evaluation, European Medicines Agency (EMA)

Dr ALTERI started her presentation by introducing the so-called ‘mandatory scope’ of the centralised procedure, handled by EMA in London. The centralised procedure is compulsory for those diseases that most significantly affect the European population, such as HIV/AIDS, cancer, diabetes, neuro-degenerative diseases, auto-immune and other immune dysfunctions, and viral diseases.

Dr Alteri stated that EMA believes that the centralised procedure offers many benefits for EU citizens, as medicines are authorised and product information is made available for all citizens at the same time. In addition, a centralised safety monitoring model has been put in place. Dr Alteri presented the number and type of medicines approved by EMA in 2015, which include medicines considered to be breakthroughs or very important treatments. She stressed that EMA’s centralised procedure lays the foundation for further positive action in access to, and availability of, medicines.

Next, Dr Alteri described the typically long road to bring medicines to patients, pointing out that EMA’s role in the assessment and approval of medicines is only one small part of the overall process. It is important to ensure that the right information is gathered during the long development phase, and she suggested that EMA should perhaps have a broader role in the overall journey that medicines go through. Dr Alteri set out the roles of the different entities intervening in the HTA process, each of whom has a different interest. For example, while the regulatory agency will focus on the benefits and risks of the drug, those paying will want to know about the health and cost consequences relative to other interventions in a defined group of patients. Moreover, the prescribers will be interested in knowing how the drug performs compared to other interventions and the patient will consider if he/she is willing and able to pay for the treatment.

In order to answer all of these concerns, EMA and those bodies responsible for the Health Technology Assessment need information on elements such as the comparator, standard of care, duration of the trial, patient population to be included pre- or poste-marketing, survival
and quality of life. In order to facilitate the data gathering, EMA started a parallel EMA-HTA scientific advice process. Here, it meets companies, developers and SMEs, in a bid to address its own, as well as its partners’ needs. To-date, 63 parallel EMA-HTA scientific advice procedures have taken place with HTA bodies from 12 Member States, and that the number of such procedures are increasing.

Dr Alteri then presented another initiative, PRIME ‘PRIority Medicine’, that EMA believes may facilitate patients’ access to medicines. PRIME is not a new authorisation process but, rather, builds on an existing framework to foster the development of medicines with major public health interest. It is needs driven, as it addresses unmet medical needs. PRIME fosters early dialogue in order to facilitate robust data collection and high quality marketing authorisation applications, thereby speeding up evaluation. PRIME was launched in March 2016 and the first numbers look promising. For example, SMEs have taken up EMA’s offer of early dialogue before starting full-fledged medicine development, and the therapeutic areas targeted are quite broad (e.g. oncology, infectious diseases, vaccines, haematology-haemostaseology, etc.). She outlined the first six products granted eligibility, each of which addresses serious diseases such as Ebola, Alzheimer’s disease, leukaemia, etc.

Dr Alteri concluded by stating that EMA’s approaches to medicines and authorisation procedures have proven valuable and that they benefit all European patients. EMA is aware of the importance of the steps that follow authorisation at national level and understands that it is essential to meet the needs of HTA as well as regulators throughout the data gathering phase. Finally, Ms Alteri stated that it is a priority for EMA to encourage Member States to support the development of medicines that benefit public health. She believes that the PRIME scheme and other early dialogue and support tools will support achieving this goal.

1.4.2. Health Technology Assessment: A Perspective from Belgium

*MEP Mr José Inácio FARIA, Shadow rapporteur*

Mr Faria introduced the next presentation by pointing out that access to innovative medicines requires clinical evidence to justify public coverage. Health Technology Assessment (HTA) has proven to be an efficient tool to provide decision makers with the evidence they need and, as such, improves access to medicines.

*Dr Raf MERTENS, Director of the Belgian Health Care Knowledge Centre (KCE)*

Dr Mertens explained that the Belgian Health Care Knowledge Centre (KCE) is one of 10 Health Technology Assessment (HTA) agencies in Europe and is actively involved in the EUnetHTA Joint Action. KCE’s area of intervention is the clinical development pathway of drugs, in particular examining the external validity, comparative effectiveness, cost-effectiveness, and budget impact of drugs in a coordinated way with other European HTA agencies.

Dr Mertens highlighted the major challenge for KCE in respect of the avoidable waste or inefficiency in biomedical research. In particular, recent scientific articles demonstrated that there is often a discrepancy between the focus of medical research and current public health needs. As a result, considerable resources are employed to develop drugs that will not meet patients’ needs. This stems from inappropriate design of clinical trials and inefficient research analysis, as well as selective reporting. For this reason, Dr Mertens explained, HTA agencies

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like KCE have to carry out ‘detective work’ to find enough data to close the gap between biomedical research and public health needs. Another issue is the lack of public health decision makers, which leads to products that are not driven by medical needs, but that are based on inadequate trial designs and are highly priced.

Dr Mertens explained that access to medicines that do not match patient needs is not satisfactory and it also means a lost opportunity for research. He mentioned several successful initiatives that can be used to enhance the accessibility of well-developed medicines. These include adaptive pathways, managed entry agreements and access coverage, with or without evidence generation. However, it is important to guarantee the accessibility of the medicines while preventing some of the safety risks to which patients might be exposed. In order to get market authorisation for a drug, a randomised controlled trial (RCT) is needed, to reduce the bias when testing a new treatment. Coverage can only be obtained if efficacy is demonstrated. However, RCT design is usually avoided, which creates risks for patient health.

Dr Mertens described some of the new developments witnessed by HTA agencies in Europe. One of the most important is the growing interest in setting up non-commercial trials to investigate research questions that are of interest to society, but which will never be answered by industry. For instance, such research questions could focus on the repurposing of older drugs, drugs in paediatrics and for orphan diseases, medical devices including diagnostics, and pragmatic comparative effectiveness trials. In particular, these latter trials are designed to evaluate the effectiveness of interventions in real-life routine practice conditions.

Dr Mertens presented KCE’s recent, publicly funded trial programme, which is collaborating with others across the EU, such as the British National Institute for Health Research (NIHR). The programme allows KCE to contribute to capacity building in the field and to strengthen the trial capability of Belgian universities and hospitals, which will bring benefits for society.

KCE is aware of the increasing price of drugs and the pressures this brings to bear on budgets. Dr Mertens referred to studies that demonstrated the exponential increase in drug costs, referring to a publication from the Italian Drug Agency32 on the case of ‘sofosbuvir’, a medicine with an unsustainable cost33. Dr Mertens emphasised that numerous patients have been denied access to this drug due to its exorbitant and unjustified price.

Finally, Dr Mertens outlined the process by which a price is defined for a drug and the role played by HTA agencies in this. He concluded that in order to bring prices down, waste must be reduced by focusing on the real public health needs, as well as emphasising safety, efficacy and therapeutic added value, and publicly funded regulatory systems.

1.4.3. Prices and Affordability

Dr Fernando LAMATA, Expert on Health Care Policies

Dr LAMATA opened by stating that while humans may not have any influence over things such as the rotation of the earth or the sun, it is possible to influence the affordability of medicines. People are dying prematurely, he said, because they cannot afford the high costs of medicines. This injustice violates human rights but it is within human power to change it.

33 Pani, L., 2016, Sustainable Innovation, Medicines and the Challenges for the Future of our National Health Service, EDRA.
The Health Council Conclusions of 17 June 2016 noted with concern that patients’ access to effective and affordable medicines is endangered by high and unsustainable prices. He explained that pharmaceutical corporations can demand high prices because their governments have granted them a monopoly through their patent and data exclusivity systems. Dr Lamata specified that the data protection system was granted by governments to pharmaceutical companies in order to allow them to recover their investments in R&D. The patent system, however, allows pharmaceutical industries to charge higher prices, thereby limiting the accessibility of medicines. Problems arise, he said, when companies charge huge prices that are not justified by costs and governments and the European institutions fail to react.

The initial arguments put forward by companies for imposing high prices, such as price of previous treatments and supposed savings, are no longer valid, as external reference pricing, differential prices and HTA have made it possible to obtain more convenient prices. But, Dr Lamata explained, prices remain exorbitant because they are calculated from the maximum possible price that the market would bear. Similarly, payers use different mechanisms to obtain better purchasing conditions for reimbursing medicines, discounts, cost-effectiveness analysis, risk-sharing, etc. These and other mechanisms, like tenders, are useful to reduce excessive pharmaceutical expenditure. However, the final price will not be fair as long as the starting negotiating point is not fair. Consumers are therefore obliged to accept an abusive price.

Dr Lamata explained the method of pricing, using the example of hepatitis C treatment. Without patent systems but with effective competition, prices would be between EUR 160 and EUR 300 per treatment. When patent protection prices are considered, as well as manufacturing and R&D costs, the total would be between EUR 300 and EUR 600 per treatment. Unfortunately, Dr Lamata explained, the reality is that prices are set at the maximum that the market can bear. Companies demand an exorbitant starting price and negotiate favourable prices with governments by offering them striking reductions. Prices remain exorbitant, resulting in price increases of around 6,000%. Dr Lamata added that the strategy of the pharmaceutical industry is to convince the stakeholders, such as doctors, ministers, patient organisations and the public, that new drugs are very expensive. The focus of the discussion therefore turns to possible mechanisms to pay for the drugs, which is a good framework for companies, but the wrong approach for health systems and patients.

Dr Lamata pointed to the importance of understanding the ‘financialising’ of the pharmaceutical sector. In this context, medicines are looked upon as financial products, with aspects such as affordability and human rights no longer being considered. Instead, the logic of drug prices becomes the logic of financial markets and profits. Figures suggest that net margins in the pharmaceutical industry are around 20% of sales, while in other industries, net margins are around 5% of sales. This amounts to 15 differential points, which translates into a lot of money.

Dr Lamata pointed to the compensation of CEOs in pharmaceutical companies as further evidence for the financial focus to drug development. In 2014, he said, the 10 most highly paid CEOs of pharmaceutical companies received between EUR 13 million and EUR 140 million per year. These figures are not comparable with the average wages in the Member States, which range from EUR 4,000 to EUR 14,000 per year. He added that sales from
pharmaceutical companies in EFPIA countries amounted to EUR 181 billion in 2014, while manufacturing costs are estimated at 21% of sales, according to an enquiry report of 2009.35

Dr Lamata presented further figures to support the idea that government-granted monopolies grant an unbalanced bargaining power that is being abused by pharmaceutical corporations. He suggested three parallel options to address this situation: a fair negotiation process; the use of compulsory licence; and the development of a new model of pricing which de-couples R&D funding from pricing. An agreement should be reached, he said, between governments and pharmaceutical companies on a reasonable initial save price for patents and medicines, based on accurate costs. This would allow for fairer negotiations, whereby discussion could focus on reimbursement and payment mechanisms. If this is not deemed possible, governments should seek to regulate the issue through compulsory licences and limiting data exclusivity. Governments should be supported by the EU institutions during such processes.

Next, Dr Lamata presented an alternative R&D strategy to deliver affordable treatments for hepatitis C patients, based on the cost of production calculated by the 'Drug for Neglected Diseases Initiative’ (DNDi).36 This regime (sofosbuvir and ravidasvir), if approved, will be priced at less than EUR 261 per treatment course.

Dr Lamata concluded by stating that discussions of the price of medicines have, for years, taken place within the framework designed by pharmaceutical corporations. It is now time to change this framework and think from a different perspective, one which is based on human rights, and considers medicines as public goods rather than financial products.

1.4.4. Questions & Answers

MEP Mr José Inácio FARIA, Shadow rapporteur

Before opening the floor for questions, Mr Faria invited Dr Christine DAWSON, Director of the European Social Insurance Platform (ESIP), to speak briefly on sustainable access to innovative pharmaceuticals.

Dr Christine DAWSON, Director of the European Social Insurance Platform (ESIP) and Coordinator of Medicines Evaluation (MEDEV) Committee

Dr DAWSON thanked Mr Faria for the opportunity to present the views of the European Social Insurance Platform (ESIP), as well as the one of the International Association of Mutual Benefit Societies (AIM), which represents the non-profit healthcare payers in Europe. The goal of the AIM is to defend access to healthcare through solidarity-based and not-for-profit healthcare coverage. The two organisations, ESIP and AIM, have published a joint position paper on access to innovative medicines, which covers many of the topics discussed during the workshop. She also expressed her wish that elements of the paper would be reflected in the European Parliament’s own-initiative report on improving access to medicines in Europe.

Dr Dawson highlighted one specific issue covered by the ESIP-AIM joint paper, namely, the importance of improving transparency across all elements of the access pathway. She stressed that greater transparency would facilitate better access to innovative high-quality and value-added medicines. This would include, firstly, greater transparency about public and private investment in R&D, which ultimately will need to be reflected in negotiating fair prices. Secondly, transparency about HTA tools and processes would enhance both industry and public trust and confidence, as well as improving cooperation and exchange of information between Member States’ competent authorities. Thirdly, transparency concerning clinical trial data, including negative data, and transparency in pricing and reimbursement mechanisms, including managed entry agreements (MEA).

Dr Dawson emphasised that ESIP and AIM are taking note of EU level reflections on potential models of flexible pricing and reimbursement. The organisation believes that an adaptive pathway approach to pricing and reimbursement (involving early dialogue, conditional reimbursement and flexible pricing) could be a way of sharing the economic risk between the company and the payer, thereby allowing patients earlier access to promising new medicines. Such a mechanism, however, would be unacceptable without transparency and strict regulation. Transparency around the selection criteria (the suitable candidate medicine), the applicable exit criteria, the data collected, data ownership, and the managed entry agreements (MEA) are all of paramount importance.

Dr Dawson concluded by stating that both ESIP and AIM remain available to discuss any of these issues in support of the European Parliament’s own-initiative report. She also hoped that the Parliament would support the European Council Conclusions adopted in June40.

The floor was then opened for questions from the audience to the speakers of Part 3 of the workshop.

Mr. Damian CABALLERO (Plataforma de Afectados por Hepatitis C), started by stating that his organisation has achieved a hepatitis C plan after a long struggle but remained unhappy with the exorbitant prices that jeopardise the national healthcare system. Such high prices do not allow for the development of sustainable national healthcare services, which then creates further problems in access to medicines. He pointed out the importance of addressing the lack of information and transparency in order to change the system. He stated his belief that the law on patents and intellectual property should be changed. He concluded with the suggestion that organisations fighting for changes in access to medicines should have a coordinating body enabling them to work together.

Ms Auken, MEP, explained that it is important to contest pricing, but this is, unfortunately, only addressed by EMA through adaptive pathways. She also stressed that NGOs should continue their fight to improve access to medicines.

Dr Alteri stressed that the system has to serve the patient and that it must be certain about the effects, safety and affordability of medicines. She insisted that EMA is looking for every possible means to improve the system, and patients remain their utmost priority.

Mr Hammerstein (former MEP) expressed his concern that Europe is in crisis because it may be perceived as a barrier to the daily needs of citizens. Intellectual property rights laws and data exclusivity laws might be seen as barriers, preventing access to cheaper treatment. Disadvantages exist in countries like Romania and Greece because Europe imposes obligations that result in exclusion of treatment for millions of people. Mr Hammerstein asked 40 Council Conclusions on strengthening the balance in the pharmaceutical systems in the EU and its Member States, 17/06/2016, available at: http://www.consilium.europa.eu/en/press/press-releases/2016/06/17-epsco-conclusions-balance-pharmaceutical-system/ (accessed August 2016).
if MEPs could suggest other concrete ideas, aside from transparency, that would remove the barriers that create high pricing.

Mr Faria responded with an assurance that MEPs are very much involved in discussion on these matters. The topic is debated almost daily in the group of the Alliance of Liberals and Democrats for Europe, he said.

Ms Ancel.la SANTOS (Health Action International) asked Ms Alteri if PRIME focuses more on the pre-market phase than the post-market phase. She highlighted the importance of pharmacovigilance and stressed that certain companies do not always seem to honour their pharmacovigilance commitments. She asked what EMA had done to ensure that pharmacovigilance commitments are honoured by companies.

Dr Alteri replied that pharmacovigilance is a priority for EMA. Supporting companies in early development does not mean that they stop receiving support when planning the post-authorisation phase in all pharmacovigilance activities.

1.5. Part IV: Proposals to improve access to medicines

1.5.1. Auctions of pharmacy-dispensed drugs

Mr Aquilino ALONSO MIRANDA, Health Minister, Andalusian Government

Mr ALONSO MIRANDA started by introducing Andalucia, a province of Spain with eight million inhabitants and a size of 87,000 km2. With such a large area and population, if it were a country, it would be the fourteenth largest in the EU. Andalucia has 48 public hospitals and employs over 95,000 healthcare professionals. Its health budget for 2016 is over EUR 8,800 million, of which 26.6% is ring-fenced for pharmaceutical expenditure.

In Spain, healthcare responsibilities have been transferred from the State to the Spanish provinces. This has allowed Andalucia to take a series of decisions since the 1990s aiming to guarantee access to medicines and innovation for all its residents. One of the first measures was the introduction of mandatory prescription by active ingredient, as recommended by the WHO and intended to guarantee the quality of prescriptions. Andalucia was the first region in the world to introduce this approach (in 2001), and around 93% of prescriptions are currently issued on the basis of this active principle.

Mr Alonso Miranda explained that, before this procedure started, pharmacies could choose the brand to be dispensed for each prescription, allowing pharmacies to negotiate discounts with pharmaceutical companies. The Andalusian Public Health Administration decided to transform these discounts into public health system savings, reinvesting the profits in the population and supporting a sustainable healthcare system. In return, companies obtain exclusive use of their products for two years, which commits them to the permanent supply of their products. The quality of medicines is also guaranteed, as medicines must first be approved by the Spanish Medicines Agency.

Mr Alonso Miranda considered this a transparent, competition-friendly process, where businesses provide a direct contribution to the Andalusian health system, and the pharmaceutical offices dispense the drugs when they are prescribed under the active principle. However, while the process results in savings supporting pharmaceutical innovation, it does not tackle the existence of unsustainable prices for public health systems.

He emphasised that Andalucia supports rational drug use and that this has been leading to significant savings. The Andalucian administration has put in place various tools for decision taking on rational drug use, including a set of recommendations for efficient drugs use in healthcare facilities, drawn up together with scientific associations. In addition, specific goals
on drug selection are set out in clinical management plans, while public contracts have been adopted for dispensation by the pharmacy services of hospitals. A central committee is in place to optimise pharmacotherapy and the public health system plays a key role in selecting the best and most efficient medicines and ensuring that the procedure is fair. Finally, Mr Alonso Miranda highlighted that the Andalusian Administration also supports the Documentation Centre and the HTA centre in Andalucia.

Mr Alonso Miranda stressed the importance of access to innovation, particularly country-wide access to e-prescriptions. The use of e-prescriptions allows health professionals, upon the patient’s authorisation, to have access to all medicines prescribed to the patient, and to change the prescription whenever necessary, using the active principle for another commercial brand.

He concluded with some strategic ideas on ensuring efficient public selection of the drugs to be dispensed among citizens. Firstly, he reiterated that such an approach takes time, and it is impossible to achieve the results in a short period of time. Any measure adopted must, therefore, have a long-term perspective. There is a need for more transparency, he said, and the involvement of professionals is crucial to ensure quality-oriented policies that the population believe in. This support is required if health systems are to maintain their credibility, as well as helping to ensure access to innovative medicines for all.

1.5.2. The role of the Innovative Medicines Initiative (IMI) in research, development and innovation in medicines

Mr Pierre MEULIEN, Executive Director, IMI

Mr MEULIEN explained that IMI is a large public-private partnership that brings together EFPIA companies, academics and SMEs. It is a large programme, spanning from 2008 to 2024 with a EUR five billion budget, half of which comes from the European Commission and half from the private sector. During the second phase of IMI, the initiative funded universities, SMEs, mid-sized companies, patient groups, HTA bodies, etc. Since 2008, IMI has been able to build an international, cross-sector community, which currently involves over 9,000 researchers working for open collaboration, improved R&D productivity and innovative approaches to unmet medical needs.

Current drug development is inefficient, risky, time-consuming, complex and expensive, Mr Meulien said. Clinical trial designs are not always optimal and regulatory pathways are not always used efficiently. IMI has worked to address these challenges. Firstly, through building a neutral platform where all key players can come together, sharing risks and maintaining the focus on patient needs. This has increased efficiency, reduced duplication of effort and timelines, and integrated the latest science and evidence. He indicated that IMI has recently performed an independent social and economic impact study on just the first 10% of the IMI-1 investments. According to this report: ‘The IMI 1 projects reviewed were not designed to directly bring new medicines to market. Rather, they will impact on new product development by acting on the medicines development process itself, usually in particular disease areas’.

Mr Meulien described the IMI-1 outputs, including new tools to facilitate drug development, clinical studies, over 1,600 scientific publications, 13 spin-off companies and over 460 biological marker candidates for better diagnosis and treatment. IMI is also involved in projects on patient safety and medicine development. One of the main challenges in drug development is to pick up on toxicity issues early in the process. This often happens at a late stage, when a lot of money and time has already been spent. To address this issue, IMI brought together public and private sector stakeholders, in order to be able to combine toxicity data both from companies and academia. This allowed them to build some simple tools to detect toxicity issues at an early stage. As an example, he described the ‘e-tox’
project, which built a database based on pharmaceutical and public data, and which has resulted in a reduction in animal testing.

Mr Meulien then focused on the development of new medicines and the development of tools to study diseases and make new discoveries on the underlying causes of diseases. He introduced the ‘EU-AIMS’ project on autism. This project has so far discovered new insights into underlying causes (e.g. genetics) of autism, has shown that brain changes associated with autism could be reversed, and studied gender differences in the brains of men and women with autism. The project involves many patients working directly with the regulators, and it has been recognised globally as a flagship project in autism.

A final challenge raised by Mr Meulien was the screening of chemical compounds in the hunt for molecules that could be used in drug development or could be potential drugs themselves. The storage of all of this information and data has traditionally been done behind closed doors. The project ‘European Lead Factory’ brings together a state-of-the-art compound collection and screening centre, which is delivering results for academics, SMEs and pharmaceutical companies. It allows the expansion of drug discovery efforts and is showing promising results against some viral diseases, such as dengue, and also in the cancer field.

1.5.3. The Council Conclusions on strengthening the balance in the pharmaceutical systems in the EU

Mr Maurice GALLA, Senior Policy Officer/EU Presidency Coordinator, Directorate for Pharmaceuticals and Medical Technology, Ministry of Health, Welfare and Sports, the Netherlands

Mr GALLA presented the priorities set during the recently finished Dutch EU Presidency, whose key theme was to strengthen checks-and-balances in pharmaceutical systems. The first priority was to focus on improving voluntary cooperation and exchange of information on pricing and reimbursement between Member States, who tend to deal with similar issues individually. However, he also stressed the importance of finding a good balance between national decision-making and cooperation among Member States. The second priority was to support timely access to new essential medicines by clarifying conditions and exit options. The third was to initiate a debate on the unintended effects of current incentives in EU pharmaceutical legislation, and their impact on innovation and costs. The last priority was to encourage a strategic debate on cooperation on future challenges and directions for pharmaceutical policy in the EU.

Mr Galla then explained why the pharmaceutical system was a priority for the Dutch Presidency. He commended the current EU pharmaceutical system, stressing that it is innovative and provides safe and effective medicines for a majority of citizens. There is also a level of investment in the development of new pharmaceuticals for rare diseases and paediatrics. The ability of Member States to decide for themselves which pharmaceuticals they wish to pay for is a positive aspect, allowing for adjustments according to national needs. There are, however, concerns about the current system. Firstly, the increasing mismatch between marketing authorisations topped up by incentives to support innovation and affordability of, and access to, the final product. Secondly, there is an increasing focus within industry on the development of (new) pharmaceuticals for smaller patient groups with high earning potential, and not necessarily on broader unmet medical needs. Thirdly, certain countries, especially smaller ones, are unable to ensure the availability of essential medicines due to unaffordable high prices, withdrawals due to low revenues or (too) small markets. A fourth concern is that governments act individually, while business acts globally, causing an information asymmetry. And finally, public investment in R&D does not always benefit public interest and citizens end up paying twice.
Mr Galla highlighted the strong interconnections between the three corners of the system (regulation, pricing and market), saying that there is a need to look at the system in an integrated way. If governments do not take responsible decisions about reimbursements, for example, this will directly affect availability and new products coming onto the market.

During the Dutch Presidency, informal discussions between Ministers concluded that imbalances must be tackled, and voluntary cooperation efforts on pricing and reimbursement should be increased. However, such voluntary cooperation should have a clear added value and decisions on pricing and reimbursement should remain a Member State prerogative. There is a need to increase knowledge of the (un)intended effects of intellectual protection to ensure that it benefits public health, all the while ensuring that tackling the unintended effects of incentives does not discourage innovation.

Mr Galla introduced the Council Conclusions on strengthening the balance in the pharmaceutical systems in the EU and its Member States, adopted under the Dutch Presidency. The Council Conclusions make a political statement, recognising the imbalances in the system and aiming to ‘rebalance’ the system to make it work as it was initially intended. As part of the process, Member States agreed that they would invest more in voluntary cooperation on pricing and reimbursement (such as the BENELUX cooperation), collaborate across the system and ensure follow-up actions.

Mr Galla concluded by explaining some of the actions required to improve the EU pharmaceutical system. Firstly, Member States must invest in cooperation work through joint activities, information sharing, joint negotiations with selected countries and cooperation on HTA, including through EUnetHTA. Moreover, the Commission and Member States must strengthen dialogue and invest in R&D to address unmet needs. And finally, for the Commission, it is important to create an overview of the pharmaceutical system and analyse the measures, individually and in combination, to see how the interact and support innovation.

1.5.4. The European Commission agenda on access to medicines

Mr Andrzej RYS, Director, Health Systems, Medical products and innovation, DG SANTE, European Commission

Mr RYS began with a brief overview of the common challenges faced by the current pharmaceutical system (increasing consumption of medicines, proliferation of high costs, etc.). He highlighted that the debate about access to medicines - the proportionality of prices with health benefits, the efficiency of pharmaceutical spending in terms of affordability and access to effective treatment for the patients - is happening at national, EU and international level, adding to the complexity of the debate. The discussion encompasses three cornerstones, each of which must be taken into account: access to affordable medicines for patients, value for money for patients and payers, and the sustainability of health systems.

Mr Rys then explained the role of the Commission and the actions it is undertaking. Firstly, it facilitates the development and authorisation of innovative medicines for unmet medical needs, for example through PRIME and the adaptive pathways approach by EMA. Secondly, it has established the Expert Group STAMP (Safe and Timely Access to Medical Products), whose goal is to identify ways of optimising the use of existing regulatory tools. Together with EMA, the Commission encourages parallel and coordinated work between innovators, HTA and regulators (e.g. the EUnetHTA joint action).

Following on from the June 2016 Council Conclusions, the Commission is committed to assessing the impact of the Social Protection Committee (SPC), data protection and market exclusivity incentives from the health perspective, in particular their effect on innovation,
availability and accessibility. The methodology and timeframe will be proposed by the Commission by the end of 2016.

There are two areas of medicine development where the Commission plays a specific role. Firstly, in the area of orphan medicines, where a new notice was launched (to replace the previous communication) to make the orphan designation process more effective as a tool. It aims to encourage R&D for orphan medicines and to ensure that the best treatments receive a designation. The second area of development concerns paediatric medicines. The Commission is currently preparing the second report on the impact of the Paediatric Medicines Regulation, which is supported by an analysis of the public health case, as well as the economic impact of the Regulation.

Finally, Mr Rys stressed the importance of HTA in assessing the added value (relative effectiveness) of a given health technology over and above existing ones. HTA gives a clearer idea about pricing and allows Member States to take rational decisions about budgets. A first initiative, the HTA network, allows the Member States and the Commission to develop policy and strategic cooperation. The second initiative, the EUnetHTA Joint Action, allows for more scientific and technical cooperation. Mr Rys ended with his hope that all of these tools will allow for a more sustainable European pharmaceutical system.

1.5.5. Questions & Answers

Ms Elsa TOBEÑA SANTAMARIA (Plataforma de Afectados por Hepatitis C) raised the issue of fair pricing, highlighting the need to remember how much a human life is worth. The people must be considered first, as patients and not clients, before issues like patents and licensing are discussed. The lives of patients should play a more important role than the arguments of pharmaceutical lobbyists.

Juan José RODRÍGUEZ SENDÍN (Organización Médica Colegial de España) asked if the mobilisation of the government and European Union in political terms is currently sufficient to solve the problems.

Ms Cabezón agreed that governments and the EU are not yet sufficiently mobilised, stating her belief that more can be achieved through increased collaboration. Reimbursement systems can be designed by Member States, while matters such as authorisations and analysis criteria could be placed under European regulation. The Parliament will try to push this topic forward to create the political momentum to make the right decisions.

Ms ’t Hoen asked Mr Rys if the Commission was considering implementing the action list outlined in the Council’s Conclusions, and if it envisaged holding public hearings. Such a move, she said, would prove to many that the topic is being taken seriously.

Mr Rys responded by outlining possible initiatives in the field of HTA that would allow for new improved regulation approaches, including, perhaps, public hearings. It is important, he said, to find ways to enrich the current knowledge about the market and fill in any potential data gaps. Member States can provide this information for studies, which will in turn allow for more public input.

Ms Auken, MEP, asked Mr Meulien who, in the IMI initiative, is responsible for establishing budget priorities, given that he had not raised the matter in his presentation.
Mr Meulien explained that everything done by IMI is part of a strategic research agenda, which was developed and agreed by a large group of stakeholders. All of the work is in line with the 2013 WHO document on priority medicines⁴¹.

Ms Auken, MEP, then expressed concern about the lack of safety in adaptive pathways that circumvent the issue. She wondered whether public procurement directives might be helpful. Finally, she expressed her disagreement with the Social Protection Committee, which allows industries to prolong their patents by an additional five years, thereby preventing generic medicines from entering the market.

Mr Meulien responded that IMI is attempting to build an adapted procurement model which would facilitate the buying process of vaccines and antivirals. Most Member States have committed to participate in this system, despite its complexity. There are new rules in the joint, public and innovative procurements which have to be used by Member States and these become quite complex cross-border. With regard to the SPC, the initial aim, he said, was to help to retain the best innovative medicines on the market, however, if this is not used properly it will be reassessed.

Dr Lamata stressed the importance of reducing the time for new medicines to enter the market. Reducing the time from ten to five years, while keeping safety and effectiveness, would be a great improvement in terms of accessibility. However, this means that patent protection time would have to be diminished, reducing income and rewards for the researchers.

Aliénor DEVALIÉRE (Médecins Sans Frontières, MSF) stated that she feels R&D is failing in terms of providing affordable and suitable medicines. She highlighted that 40% of R&D costs are coming from the public sector and from patients. She asked Mr Meulien how IMI ensures that large amounts of money are reflected in final projects of development and the price of medicines.

Mr Meulien stated that the increase in efficiency of the drug development process, which includes a large proportion of public money, should be reflected in the price of medicines. In principle, it all depends on how much money actually goes into each stage of the process. If the process if efficient, then the advantages should be reflected onto the consumer.

Ms Iciar SANZ DE MADRID (Farmaindustria) said that her organisation is committed to the needs of both governments and patients when looking for agreements. She stressed the need for a win-win situation for all parties involved. The pharmaceutical industry is not made up of aggressive lobbies hunting for profit, she said, but, rather, it defends both the interests of the industry and the patients. It is happy to take on board social programmes to help patients, however, the lobbies have nothing to do with the commercial policies as they are individual in-house decisions taken by every company.

Mr Chase PERFECT (Coalition PLUS) referred to the future of the orphan drug programme, advising caution in ‘doubling down’ on the current model. He pointed to a recent increase in orphan drug designations in the US, although it is not clear whether this increase is due to innovation or adaptation in registration strategies. He was wondering if the Commission is looking into the fact that stratification of diseases is responsible for increases in orphan drug approvals, rather than actual innovation.

Mr Rys responded by confirming that the Commission is indeed looking into this issue.

1.5.6. Closing remarks by the Chair

**MEP Mr José Inácio FARIA, Shadow rapporteur**

Mr Faria thanked all of the attendees for their participation and expressed his hope that many more such workshops would happen in the future. He thanked Ms Cabezón in particular for organising the event. Finally, he highlighted the importance of transparency standards and his direct involvement in the Transparency Directive which was, unfortunately, sidelined.

**MEP Ms Soledad CABEZÓN, Chair, ENVI Health Working Group**

In closing, Ms Cabezón thanked all of the participants for their contribution to the rich debate. She highlighted the importance of developing more sound regulations and improving the system of access to medicine as a matter of human importance. Member States have an obligation to introduce this fundamental right and there is a general concern among countries who are unsuccessfully trying to get medicines to patients. The market must be balanced, of course, and the European pharmaceutical industry will continue to be competitive, but society must place the patient at the centre of its decision-making. Ms Cabezón reiterated her belief that if all stakeholders work together they will be able to draw up a plan to develop a more adapted system.
ANNEX 1: PROGRAMME

WORKSHOP

EU Options for Improving Access to Medicines

14 July 2016 from 09.00 to 12.30
European Parliament, Altiero Spinelli A3G-2, Brussels

Organised by the Policy Department A-Economy & Scientific Policy for the Committee on the Environment, Public Health and Food Safety (ENVI) Working Group Health

AGENDA

09.00 - 09.10
Opening and welcome
MEP Ms Soledad CABEZÓN, member of the ENVI Health Working Group

Part 1

Access to medicines from different points of view: Analysis of the current situation

09:10 -09:15
Pharmaceutical industry of medicines produced in Europe
Mr Paul VAN HOOF, European Federation of Pharmaceutical Industries and Associations (EFPIA)

09:15 – 09:20
Medicines for Europe, the voice of the generic, biosimilar and added-value medicines in Europe
Mr Adrian VAN DEN HOVEN Director General, Medicines for Europe

09:20 – 09:25
NGOs and access to medicines
Mr François FILLE, European Advocacy Coordinator, Médecins du Monde

09:25 – 09:30
Patients associations and access to medicines
Ms Kaisa IMMONEN, Director of Policy, European Patients’ Forum (EFP)
Part 2

Functioning analysis of pharmaceutical systems in the EU

09:30 – 09:40
Intellectual property law system applicable to medicine
Ms Ellen ’t HOEN, Medicines Law & Policy and Global Health Unit, University Medical Centre, University of Groningen

09:40 – 09:50
European Systems for prices and reimbursement of medicines
Prof. Dr Lieven ANNEMANS, Health Economics Unit, Department of Public Health, University of Ghent

09:50 – 10:00
Competition enforcement for pharmaceutical products
Mr Dirk VAN ERPS, Head of Unit Antitrust: Pharma and Health Services, DG COMP

10:00 – 10:25
Questions & Answers for parts 1 & 2

Part 3

Authorisation procedures and Systems for pricing and reimbursement

10:25 – 10:35
Role of EMA in authorising and supporting access to new medicines
Ms Enrica ALTERI, Head of Human Medicines Evaluation, European Medicines Agency (EMA)

10:35 – 10:45
Health Technology Assessment: A Perspective from Belgium
Dr Raf MERTENS, Director of the Belgian Health Care Knowledge Centre (KCE)

10:45 – 10:55
Prices and Affordability
Dr Fernando LAMATA, Expert on Health Care Policies

10:55 – 11:20
Questions & Answers

Part 4

Proposals to improve the access to medicines

11:20 – 11:30
Auctions of pharmacy-dispensed drugs
Mr Aquilino ALONSO MIRANDA, Health Minister, Andalusian Government

11:30 – 11:40
The role of the Innovative Medicines Initiative (IMI) in the research, development and innovation in Medicines
Mr Pierre MEULIEN, Executive Director, IMI
11:40 – 11:50
The Council Conclusions on strengthening the balance in the pharmaceutical systems in the EU
Mr Maurice GALLA, Senior Policy Officer/EU Presidency Coordinator, Directorate for Pharmaceuticals and Medical Technology Ministry of Health, Welfare and Sports, The Netherlands

11:50 – 12:00
The European Commission agenda on access to medicines
Mr Andrzej RYS, Director, Health Systems, Medical products and innovation, DG SANTE, European Commission

12:00 – 12:25
Questions & Answers

12:25 – 12:30
Closing remarks by the Chair
ANNEX 2: SHORT BIOGRAPHIES OF EXPERTS

Dr Paul van Hoof

Dr van Hoof has worked in the pharmaceutical Industry for 23 years, holding various roles in the Netherlands, the UK, Slovakia, and currently in Belgium as Director of European Government Affairs for GSK. He has broad experience, with an emphasis on General Management, Access, Pricing and Reimbursement of Pharmaceuticals and Health Policy development. On a personal level, Dr van Hoof is co-founder of the CEE health policy network, and is actively involved in improving the health of Roma communities living in secluded circumstances in Central and Eastern Europe. He is a member of the EFPIA working groups on Pricing and Health systems. At this workshop, Paul represents EFPIA as the Brussels-based chair of the Working Group for access to medicines.

Mr Adrian van den Hoven

Mr van den Hoven was appointed Director General of Medicines for Europe on 1 September 2013. Prior to this he was Deputy Director General of BUSINESSEUROPE, with responsibility for the International Relations and Industry departments. He worked as an International Relations researcher and adjunct professor in Italy (EUI), France (Nice) and Canada (Windsor) before joining BUSINESSEUROPE in 2003. He received his doctorate in Political Science from the University of Nice in 2000.

Mr François Fille

Mr Fille is the Europe Advocacy coordinator for Médecins du Monde/Doctors of the World International Network office. He is involved in different dossiers, including migrant health and advocacy activities linked to the EU funded European Network to Reduce Vulnerability in health. Mr Fille graduated in Political Science at the Aix-en-Provence Institute of Political Studies. He has been involved in humanitarian development work for the past 23 years, managing many humanitarian programmes, mostly with Médecins Sans Frontières but also with Action Contre la Faim and the Thai Burma Border Consortium. From 2001 to 2006, Mr Fille was responsible for MSF Belgium European domestic projects, as well as a variety of projects in the Balkans, the Horn of Africa, Asia and South East Asia. These projects also dealt with issues in access to treatment, such as the Access to Drug Campaign in China, Thailand and Indonesia. From 2011 until March 2016, he was responsible for Médecins du Monde programmes in the Democratic Republic of Congo, Morocco, Mali and Niger, and piloted advocacy activities on gender-based violence against migrants at the Angola/Democratic Republic of Congo border.

Ms Kaisa Immonen

Ms Immonen-Charalambous (MA) is Director of Policy at the European Patients’ Forum (EPF), responsible for leading EPF’s policy and advocacy work at EU level, strategic planning, policy analysis, and building positive relationships with EU institutions and stakeholders, as well as developing EPF’s policy positions in close consultation with the membership. She leads the thematic area of empowerment and patient-centred chronic disease care within EPF. Ms Immonen is a member of the European Commission’s Expert Group on Patient Safety and Quality of Care, the EMA’s Working Party with Patients and Consumers, and the PISCE Platform of Experts on Self-Care. Before joining EPF in 2010 she worked in EU health policy,
advocacy and external communications roles in both the private and non-profit sectors. Ms Immonen has a Master’s degree in International Relations (UK and Finland).

**Ms Ellen ‘t Hoen**

Ms ‘t Hoen, LLM is a lawyer and public health advocate with over 30 years of experience working on pharmaceutical and intellectual property policies. She is an independent consultant on medicine law and policy, working with a number of international organisations and governments, as well as a researcher at the Global Health Unit of the University Medical Centre at the University of Groningen in the Netherlands. She was listed as one of the 50 most influential people in intellectual property by the journal Managing Intellectual Property in 2005, 2006, 2010 and 2011.

From 1999 until 2009 Ms ‘t Hoen was the Director of Policy for Médecins Sans Frontières’ Campaign for Access to Essential Medicines. In 2009 she joined UNITAID in Geneva to set up the Medicines Patent Pool (MPP), an initiative that negotiates patent licenses with pharmaceutical companies to ensure access to affordable generic medicines for the treatment of HIV. She remained the MPP’s first Executive Director until 2012.

Ms ‘t Hoen has published widely, and is the author of the 2009 book ‘The Global Politics of Pharmaceutical Monopoly Power, Drug patents, access, innovation and the application of the WTO Doha Declaration on TRIPS and Public Health’. She is a member of the WHO Expert Advisory Panel on Drug Policies and Management, the Lancet Commission on Essential Medicines, the Advisory Board of Universities Allied for Essential Medicines (UAEM) and sits on the Editorial Board of the Journal of Public Health Policy.

**Dr Lieven Annemans**

Dr Annemans is Senior Professor of Health Economics at the Faculty of Medicine at Ghent University. He has 20 years of experience in health economics research, during which time he has published over 250 papers on health economic evaluations of prevention programmes, medicines, medical devices and diagnostics. He is currently the Past-President of ISPOR. Dr Annemans previously worked as advisor to the Belgian Minister of Health from 2001 to 2003, as well as holding the post of President the Flemish Health Council from 2003 to 2009.

**Mr Dirk van Erps**

Mr van Erps has worked with the Directorate-General for Competition of the European Commission since 1991. During his first seven years in DG Competition he worked in the Antitrust unit dealing with the pharmaceutical, agriculture and consumer good sectors, following which he spent three years on the Merger Task Force. From 2001 to 2004, he returned to the antitrust sectoral unit as the Deputy Head of Unit. After a brief period at the Energy Unit in early 2005, he became Head of Unit in the Cartels Directorate, where he managed several international cartel investigations in addition to the Forensic IT and the Inability to Pay teams. In October 2014, he was appointed Head of the Antitrust: Pharma and Health Services unit.

Mr van Erps obtained a law degree at the Catholic University of Leuven in Belgium, as well as receiving his Master’s degree in European law from the College of Europe in Bruges.

**Dr Enrica Alteri**

Dr Enrica Alteri is a Doctor in Medicine and Surgery from Universita degli Studi La Sapienza in Rome, with postgraduate qualifications from the National Cancer Institute USA and EUCOR/ECPM. Dr Alteri has many years of experience within the pharmaceutical Industry,
specifically in the areas of drug discovery, clinical safety and pharmacovigilance. Dr Alteri joined the EMA in 2012 as its Head of Safety and Efficacy. In 2013 she was appointed Head of Human Medicines Evaluation Division.

**Dr Raf Mertens**

Dr Raf Mertens graduated from KU Leuven with a degree in medicine, following which he practiced in The Kivu (Democratic Republic of Congo) for four years. From 1985 to 1997 he was responsible for the national programme for nosocomial infections surveillance and epidemiology at the Scientific Institute of Public Health in Brussels. He obtained a diploma in medical and social hygiene (1986, KU Leuven) and in epidemiology (1989, London School of Hygiene and Tropical Medicine) and was recognised as a specialist in health data management.

Dr Mertens spent four years as Coordinator of the European HELICS programme for the harmonisation of nosocomial infections registration networks. In 1997, he took charge of healthcare data analysis and feedback, and the development of (hospital) quality of care improvement programmes at the Christian Sickness Fund. From 2001, he was also actively involved in the development of the Intermutualistic Agency, treating the pooled data of all Belgian Sickness Funds.

Dr Mertens was vice-president of the National Council for Quality Improvement and a member of the Board of the Belgian Health Care Knowledge Centre (KCE). From 2006 to 2009 he led the R&D department of the Christian Sickness Fund before becoming Director General of the Belgian Health Care Knowledge Centre (KCE) at the end of 2009.

**Dr Fernando Lamata**

Dr Lamata has over 25 years of experience in planning and management of health services at regional and national level (General Secretary of Health at the Spanish Ministry; Vice-President and Regional Minister of Health and Social Services of Castilla-La Mancha; Director of the National School of Public Health; Regional Director of the National Institute of Health of Madrid; Member of Parliament of Castilla-La Mancha; Executive Vice-president of Fundación Jiménez Díaz Hospital, Madrid; etc). He has contributed to the development of health services in Spain (particularly Primary Care and Mental Health), and participated in reform processes, decentralisation and coordination of health and social systems. He was a member of the WHO Executive Board, and of the EU High-Level Group on health services and medical care. He worked in international health cooperation projects and collaborates on postgraduate education and continuous training programmes. As doctor with a speciality in psychiatry, he has also written several books on health care management. Between July 2013 and June 2016 he has been a member of the Expert Panel on Effective ways of investing in health (European Commission).

**Mr Aquilino Alonso Miranda**

Dr Alonso Miranda obtained a Master’s degree in Public Health and Health Administration from the University of La Habana. He also obtained a Master’s degree in Occupational Health and Safety from the University of Granada, and Health Economics from the Andalusian School of Public Health.

He has extensive experience in health management in Andalucia, starting his professional career in 1988 as Director of the Health District of Loja (Granada). In 1995 he became the Director of the Health District of Granada, a position he held until 2003. Between 2003 and July 2012 Dr Alonso Miranda directed the Metropolitan Health District of Granada, before
being appointed Director of Health Services at the Health Service of the Principality of Asturias (SEAPA).

In 2013 he returned to Andalucia, serving as Deputy Minister for Equality, Health and Social Policies before taking on the role of Regional Minister of Health of the Government of Andalucia. Dr Alonso Miranda has lectured on Social Medicine at the Andalusian School of Public Health and the School of Social Work of Gijón.

**Mr Pierre Meulien**

Mr Meulien is Executive Director of the Innovative Medicines Initiative (IMI), a EUR 5 billion public-private partnership between the European Union and the European pharmaceutical industry. He is responsible for the overall management of the programme, which works to improve and accelerate the entire medicine development process by facilitating collaboration between the key players involved in health research, including universities, pharmaceutical and other companies, patient organisations, and medicines regulators. Mr Meulien joined IMI in September 2015.

Before joining IMI, Mr Meulien spent five years as President and CEO of Genome Canada, where he raised significant funds for the organisation and oversaw the launch of novel projects and networks in the field of genomics-based technologies. From 2007 to 2010, he was Chief Scientific Officer for Genome British Columbia. Prior to his work in Canada, Mr Meulien served as founding CEO of the Dublin Molecular Medicine Centre (now Molecular Medicine Ireland), which linked medical schools and teaching hospitals in Dublin to build a critical mass in molecular medicine and translational research. He also worked in the private sector, with both the French biotechnology company Transgene, and with Aventis Pasteur (now Sanofi Pasteur). He has a PhD in Molecular Biology from the University of Edinburgh, working as a post-doctoral fellow at the Institut Pasteur in Paris.

**Mr Maurice Galla**

Mr Galla is the Senior Policy Officer at the Directorate for Pharmaceuticals and Medical Technology at the Netherlands Ministry of Health, Welfare and Sports. During the Dutch EU Presidency, he coordinated the various Presidency activities in the field of pharmaceuticals and medical technology. He was also part of the Presidency team in the Trilogue negotiations on the medical devices and in-vitro diagnostics regulations. He has previously worked in policy development, analysis and legislation in the Dutch health sector and health markets, as well as in the development of (good) governance and healthcare oversight policies. Mr Galla worked for six years as a seconded national expert in the illicit drug control policy coordination unit of the European Commission and was Deputy Head of International Affairs of the Trimbos Institute, the Netherlands Institute for Mental Health and Addiction. Mr Galla studied political sciences and public administration at the University of Amsterdam.

**Mr Andrzej Rys**

Mr Rys is a medical doctor specialised in radiology and public health, having graduated from Jagiellonian University, Krakow (Poland). In 2011, he became the Director for Health Systems and Products in the Directorate-General for Health and Food Safety, European Commission. Previously he was the Director for Public Health and Risk Assessment in the Directorate-General for Health and Consumers, European Commission. In 2003, he worked as the Founder and Director of the Centre for Innovation and Technology Transfer at Jagiellonian University (Krakow, Poland), following which he worked as the Director of Krakow’s city Health Department before taking up the role of Deputy Minister of Health in Poland. Mr Rys was a member of the Polish accession negotiation team.
ANNEX 3: PRESENTATIONS

Presentation by Mr Paul Van Hoof

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Our Commitment

“Reducing inequalities in health, accelerating patients’ access to innovative medicines and improving patient safety – these are our primary commitments. By working in partnership with all relevant healthcare stakeholders, we seek to develop practical solutions to make these goals a reality.”
For patients, life expectancy continues to improve, the use of innovative medicines has made major contribution to recent advances.

From 2000 to 2009, an improvement in population weighted mean life expectancy at birth of 1.74 years was seen across 30 OECD countries.

Innovative medicines are estimated to have contributed to 73% of this improvement once other factors are taken into account (e.g., income, education, immunization, reduction in risk factors, health system access).

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One example: Medicines have transformed HIV/AIDS from a death sentence to a manageable disease.

**HIV/AIDS Age-Standardized Death Rates (ASDR)**

By Country

<table>
<thead>
<tr>
<th>Country</th>
<th>Decline in ASDR (1995-2013)</th>
</tr>
</thead>
<tbody>
<tr>
<td>USA</td>
<td>86%</td>
</tr>
<tr>
<td>Spain</td>
<td>92%</td>
</tr>
<tr>
<td>Italy</td>
<td>87%</td>
</tr>
<tr>
<td>France</td>
<td>94%</td>
</tr>
<tr>
<td>Canada</td>
<td>87%</td>
</tr>
<tr>
<td>Australia</td>
<td>88%</td>
</tr>
<tr>
<td>Germany</td>
<td>82%</td>
</tr>
<tr>
<td>United Kingdom</td>
<td>73%</td>
</tr>
</tbody>
</table>

* Source: Health Advances analysis, WHO Mortality Database (accessed February 2014).
Workshop on EU Options for Improving Access to Medicines

Biopharmaceutical companies around the world have driven a decade of remarkable advances in medicines

- 2003: First vaccine for the prevention of cervical cancer
- 2004: First treatment for chronic heart failure for 50 years
- 2005: First oral drug for treating angina
- 2006: First treatment for chronic kidney disease
- 2007: First treatment for skin cancer
- 2008: First drug to treat bladder cancer
- 2009: First drug to treat diabetes
- 2010: First drug to treat anemia
- 2011: First drug to treat heart failure
- 2012: First drug to treat cystic fibrosis
- 2013: First drug to treat Crohn’s disease
- 2014: First drug to treat brain cancer

An improved understanding of disease, leading to personalized medicines have resulted in increased patient survival

Advances in personalized medicine have improved the outlook for patients with blood cancers in Europe

Improved Understanding of the Disease

- 60 YEARS AGO: "Disease of the blood"
- 50 YEARS AGO: Leukemia, Lymphoma
- 40 YEARS AGO: Chronic Leukemia, Acute Leukemia, Pro-lymphocytes, Indolent lymphomas, Aggressive lymphomas
- TODAY: ~40 unique Leukemia types identified, ~50 unique Lymphoma types identified

A greater understanding of the molecular basis of disease has transformed what was once known collectively as "disease of the blood," into multiple subtypes of leukemia and lymphomas, which can be targeted by personalized medicines

Today, 230 medicines are in development for blood cancers in Europe

Chronic Lymphocytic Leukemia 5 year survival rates have grown to 70%
Hodgkin’s Lymphoma 5 year survival rates have grown to 80%

Note: Chart and information provided by the EU Medicines and Medical Devices (MED) Initiative, Advancing Europe’s Health, 2016-2019, www.efpia.eu

www.efpia.eu

References
Healthcare systems across Europe are facing unprecedented challenges, driven by an ageing population and an increased prevalence of chronic disease.

With over 7000 medicines in development, the new wave of medical innovation will play a key role in addressing the challenges faced by patients and healthcare systems.
Across Europe, governments and the biopharmaceutical industry are developing stability agreements that balance access to medicines, affordability and support for innovation.

Industry remains committed to engaging with payers on flexible and innovative pricing and funding models:

- Managed entry agreements for new medicines
- Outcomes-based reimbursement
- Innovative Pricing & Funding Models
Looking ahead: the need for initiatives focusing on health outcomes can improve quality of care and reduce costs

A recent study in Sweden targeting disease management found that patients enrolled in a heart failure program involving regular follow-up with specialized nurses led to improved outcomes and 30% reduced costs through fewer hospital admissions and GP visits.

‘The importance of a continuing open and constructive multi-stakeholder dialogue’

Access

Rewards for Innovation

Improved outcomes for Patients

Affordability

Sustainable Health Systems

Presentation by Mr Adrian Van Den Hoven

Access to Medicines: Stimulating Competition in Pharmaceutical Markets

Adrian van den Hoven
Medicines for Europe
14 July 2016
Healthcare Challenges

Healthcare generally
- More patients need treatment
  - Chronic diseases, cancer on the rise
- Treatment costs are increasing
- Austerity is undermining healthcare
  - Short term cost cutting vs long term efficiency models

Medicines specifically
- High cost of new drugs (HIV, HCV, oncology) creating pressure
- Generic competition is stagnating across Europe (negative growth in Spain, France, Portugal, Romania)
- Biosimilar competition is too slow (Belgium/NL low or no competition)
- Value added competition limited in most markets (governments are missing opportunities to improve efficiency in healthcare)
- Competition in specialty markets needs to involve stakeholders
- Shortages of essential medicines due to extreme pricing models (tendering)
Major EU social and economic challenges

Global spending on new brand medicines has more than doubled in 2014 and is 2.6% of global pharmaceutical spending.

![Graph showing global new brand spending growth in USD Billion (BN)]

Why Should Competition be at the Top of the Pharmaceutical Agenda?

- Generic competition & prevention are by far the biggest drivers of efficiency in healthcare.
- Biosimilar competition has the potential to massively increase access to biopharmaceuticals.
- Value added medicines can bring sustainable innovation to massively improve efficiency in healthcare.

What are the EU & Member States waiting for?
Health Council Conclusions
17 June 2016

European Health Ministers Call for More Competition in Pharmaceutical Markets:

UNDERLINES the importance of timely availability of generics and biosimilars in order to facilitate patients’ access to pharmaceutical therapies and to improve the sustainability of national health systems.

Generic medicines: A Cornerstone of Healthcare Sustainability

- 56% of dispensed medicines
- 22% of pharmaceutical expenditure
- €100bn less spending through generic competition
- +350 manufacturing sites in the EU
- High quality medicines to over 500 millions patients
- +160,000 employees
- Up to 17% R&D investment of turnover
- +100% patient access over 10 years

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Biosimilar medicines: An opportunity for access

- Large clinical experience
- 400 million patient days
- First approved biosimilar medicines
- 2006
- 1st worldwide approval
- Biosimilar vs originator
- +44% cost reduction
- 60 countries
- Cost-effective access
- Use of biosimilars
- Savings from 
  - €78 billion
  - 11.8% of EU healthcare budget

Value added medicines: Sustainable Innovation

- Retain, reinvent & optimise medicines, improving patient health & access
- Healthcare inefficiencies
- Value added medicines
- 50% of patients
- Value challenges with medication adherence

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Our Concrete Proposals to Improve Access to Medicines 1/2

- Stimulate competition with specific uptake measures for generic, biosimilar and value added medicines
- Adapt uptake measures to more complex specialty markets with stakeholder benefit sharing & involvement
- Include new generic, biosimilar and value added developments in “horizon scanning” to balance pharma markets
- Balance procurement models with supply risks to avoid a major drug shortage in Europe

Our Concrete Proposals to Improve Access to Medicines 2/2

- Adopt the SPC manufacturing waiver, improve EPO opposition procedures, and address unwarranted restrictions to competition after patent or exclusivity expiry
- Advance the regulatory efficiency to accelerate drug approvals and reduce maintenance costs
- Engage in real dialogue with medicine manufacturers (Medicines for Europe and EFPIA) for sustainable markets
- Value added medicines: creation of the specific Market Access & Pricing pathways to recognise the value of incremental innovation
Workshop on EU Options for Improving Access to Medicines

Best Practice
Dialogue on the Role of Generic Medicines for Healthcare Sustainability

Fundamed event Madrid
Stakeholders and patients

- More than 50 participants
  - 4 political parties
  - Key Stakeholders & Patients
  - National & Regional authorities

- Topics discussed
  - Off-patent Sustainability
  - Market Predictability
  - Sustainable Pricing
  - Retail tendering shortage - Andalusia

Best Practice
Cooperation with Stakeholders on Biosimilar Medicines

In partnership with governments, industry, patients and healthcare professionals, improve the education and understanding of biosimilar medicines to ensure universal access to high quality biological treatment.
**Commission Multi-Stakeholder Dialogue on Biosimilar Medicines**

Biosimilars don’t need big market share to bring savings – IMS
The European Commission has asked IMS to put together this report, drawing on data from a study published earlier this year, and updating it with more information from EU pharma trade groups. In the report, “Impact of Biosimilar Competition,” IMS identifies five key findings:

1. GROW - The impact on biosimilar competition on prices, volume and market share - updated version 2010
2. European Commission - Experts
3. The
4. 
5. patients • quality • value • sustainability • partnership

---

**How Can we Strengthen Collaboration?**

- **EUROPEAN SEMESTER**
  - Commission Expert Group on Safe and Timely Access to Medicines for Patients (“STAMP”)
  - Join forces for better balance with access to innovation and uptake of generic and biosimilar medicines
  - Stimulate off-patent contribution

- Better Access to medicines leads to better health
- Generic and biosimilar medicines uptake are the mechanism to increase access without increasing treatment costs

---

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Shortages Driven by Extreme Pricing Policies

- 93% of generic hospital medicines cost less than 1€!
- Industry participation in hospital markets is dropping to all time lows (Portugal: 15 to 2 suppliers over the last 2 years for essential drugs)
- Risks of major essential drug shortages on the rise
- German Health and Economy Ministries declare that single block tendering is a major cause of shortages (Pharmadialog)
- Hospital pharmacists consider drug shortages to be a major risk for Europe (EAHP report)

Do we react before or after a major life-threatening shortage?

Drivers of Shortages

- Quality and manufacturing
- Business Decisions
- Cost
  - Regulatory Efficiency
  - FMD
- Price
  - P&R Policy
  - Tendering
  - ERP

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Solutions to Address Shortages

1. Increased access to generic medicines
   - Decrease time to access generic medicines
   - Efficient regulatory processes
   - Sustainable price & reimbursement measures

2. Prevent Quality/Manufacturing issues

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THANK YOU!
Workshop on EU Options for Improving Access to Medicines

Presentation by Mr François Fille

NGOs & access to medicines
Médecins du Monde:
Access to healthcare and medicines in times of crisis

Françoise Swann
Workshop EU options for improving access to medicines
Brussels, July 2016

Access to medicine – HCV new drugs, the trigger

- 185 million people across the world have HCV – risk of life-threatening cirrhosis and cancer: 700,000 deaths annually
- Direct-Acting Antiviral (DAA) are a real breakthrough in HCV treatment with cure rates >90%
- Wide access would allow HCV eradication but...

<table>
<thead>
<tr>
<th>Price</th>
<th>Sofosbuvir (SOVALDI®)</th>
<th>Daclatasvir (DAKLINZA®)</th>
</tr>
</thead>
<tbody>
<tr>
<td>U.S.A.</td>
<td>$44,000 USD</td>
<td>$29,000 USD</td>
</tr>
<tr>
<td>U.K.</td>
<td>£45,120</td>
<td>£33,723</td>
</tr>
<tr>
<td>France</td>
<td>€41,000</td>
<td>€25,806</td>
</tr>
<tr>
<td>Germany</td>
<td>£44,700</td>
<td>€40,506</td>
</tr>
<tr>
<td>Greece</td>
<td>€45,000</td>
<td>€30,450</td>
</tr>
</tbody>
</table>

GILEAD'S HCV DRUG

SOFOSBUVIR APPROVED
BY THE FDA/EMA

£41,000

BUT ACCESSIBLE FOR HOW MANY?
Exorbitant prices endanger public health systems

- **Unethical price setting** based on the damage a drug can prevent instead of transparency in R&D costs and traceability of public funding of research.

- **Lead to treatment rationing** – unethical and ineffective from a public health perspective.

- **Endanger public health systems** – cost effective does not mean affordable: high budgetary impact on European national public health systems in the context of austerity and budgetary restrictions.

In France, providing sofosbuvir to all HCV patient as the annual price would cost more than the annual budget of Parisian public hospitals.

Legal tools to increase universal access to treatment by promoting generic competition

<table>
<thead>
<tr>
<th>Price (12-week course)</th>
<th>Sofosbuvir (SOVALDR®)</th>
<th>Daclatasvir (DAKUNAR®)</th>
</tr>
</thead>
<tbody>
<tr>
<td>USA</td>
<td>64,000 USD</td>
<td>63,000 USD</td>
</tr>
<tr>
<td>UK</td>
<td>48,770 €</td>
<td>33,723 €</td>
</tr>
<tr>
<td>France</td>
<td>61,000 €</td>
<td>25,600 €</td>
</tr>
<tr>
<td>Germany</td>
<td>64,309 €</td>
<td>40,539 €</td>
</tr>
<tr>
<td>Greece</td>
<td>46,990 €</td>
<td>30,450 €</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Price (12-week course)</th>
<th>Sofosbuvir (generic version)</th>
<th>Daclatasvir (generic version)</th>
</tr>
</thead>
<tbody>
<tr>
<td>India</td>
<td>288 €</td>
<td>107 €</td>
</tr>
<tr>
<td>Predicted min. costs*</td>
<td>176 €</td>
<td>14.5 €</td>
</tr>
</tbody>
</table>

**Patent opposition:** This legal mechanism has already been used by civil society in India, Brazil, United States to get abusive patents removed and to authorize the production of more affordable generic medicine.

**Compulsory license**, a public health safeguard to overcome patent barriers, used by several countries – including EU member states - to reduce prices of medicine and to improve access for their populations.

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Workshop on EU Options for Improving Access to Medicines

MdM challenges sofosbuvir's patent in Europe

- Sofosbuvir is the first new DAA on the French market and backbone of current HCV treatment.
- Patients in France were facing rationing but the government did not provide a satisfying solution.
- Sofosbuvir’s patents until 2029.
- The high price is directly connected to the monopoly situation, i.e., the patent. MdM decided to look more closely at the patent.
- Experiences in India and Egypt encouraged us to challenge the patent.
- February 11, 2015: MdM opposed the patent on sofosbuvir at the European Patent Office.

Our first goal is to show that we are facing a monopoly situation that should not even exist, because this patent is not legitimate and should not have been granted.

MdM demands French Government to issue a compulsory license

- MdM together with other civil society organizations requested French authorities to use CL to reduce the price of sofosbuvir, without success...
- A flexibility of the WTO TRIPS Agreement
- A public health safeguard
- A tool for balance between patent holder’s private right and public interest
- A leverage to reduce costs
  - Brazil CL on Efavirenz resulted in US$ billion 95 savings from 2007-2011
Doctors of the World is demanding:

- The dramatic reduction of the price of sofoevir and other DAAs so that they can be prescribed to all people living with chronic hepatitis C.
- The initiation of a public debate on drug price setting methods and alternative financing mechanisms for the research and development of new drugs.
- Transparency in the research and development costs of pharmaceutical companies and the traceability of public funding for research.

National governments should not act as if they were powerless regarding drug prices when specific legal provisions have been designed to address drug overpricing and satisfy public health needs.
Access: a fundamental patients’ right

- Treatment based on needs – not means
- Timely access to accurate diagnosis and treatment saves lives, improves health and quality of life – and benefits society
- Equity of access is not yet a reality
- EPF: "Core principles on value and pricing of innovative medicines", June 2016

Source: MS BAROMETER 2011: European Multiple Sclerosis Platform
**EPF: “Fair Access Framework” at EU level**

Agree common principles and mechanisms for encouraging Innovation

- **Pharmaceutical industry:** “walk the talk” as responsible health stakeholder
- **Member States:** political leadership & action
- **Patients:**
  - Responsible pricing
  - More transparency
  - Ethical conduct...
  - Scale up early dialogue & joint negotiation
  - Explore differential pricing...

**Patients: the vital ingredient**

- Patients are directly affected by policies
- Patients are an under-used resource: accurate assessment of (added) value is only possible with patients' involvement
  - Patient-relevant clinical endpoints, Quality of life, benefit-risk
- Need to implement systematic patient involvement across entire medicines life-cycle
  - Early dialogue, regulation, HTA, pricing and reimbursement,
    post-marketing data collection & pharmacovigilance,
  - Find or develop appropriate structures and mechanisms for this
    - both EU and national level!
In conclusion

- Core EU values: universality, quality, equity and solidarity
- Ensuring universal access to medicines is a political choice
- EU member states face common problems – must work together and in partnership with patient organisations – to find common solutions
- Measures to control costs must not (further) undermine access or stop investment in R&D
- Access to healthcare (including medicines) must be top on the EU Agenda – never more so than now!

"A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE"

THANK YOU FOR YOUR ATTENTION!

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Twitter /eupatient
Twitter /eupatientsforum

More information
www.eu-patient.eu
info@eu-patient.eu

"A STRONG PATIENTS’ VOICE TO DRIVE BETTER HEALTH IN EUROPE"
Abstract

European Union (EU) countries are struggling with the consequences of high-priced medicines for their health budgets. Making new medicines available to EU populations, including medicines recommended for use by Health Technology Assessment (HTA) agencies, has become a challenge in almost all countries. The EU is largely dependent on market exclusivity, provided through patents and other legal instruments, to fund research and development of new medicines. Pharmaceutical companies are granted patents and other forms of market exclusivity to compensate for investments they make for the development of new treatments. These incentive mechanisms affect R&D priorities and pricing of new medicines.

EU countries negotiate individually with pharmaceutical companies to bring prices down and make medicines available. Patent monopolies granted to companies put those companies in a strong bargaining position leading to suboptimal results of price negotiation.
Abstract (cont.)

Today even high income countries are seeking ways to alleviate the effects of high medicines pricing. Unfortunately in some cases this is done through rationing of medicines or delays in making them available. Rationing of expensive medicines used to be a phenomena almost exclusively to the developing world. Today it is making headlines in EU countries.

A long term solution to the problem of high medicines pricing will likely be found in changes to the way the development of new medicines is incentivised and financed.

The Council of the EU has asked the European Commission to prepare an analysis of the impact of the incentive mechanisms in EU legislative instruments on pricing and the development of new medicines.

Short term solutions will demand more proactive and transparent actions by governments.

Pharmaceutical Innovation in the EU:
Prescrire’s ratings 2000 to 2014
Percentages per category, N=1432

Addressing Challenges of access to new essential medicines for Global Health

Some Resources

- Basic information about medicines and intellectual property: [www.accessтомedicines.org](http://www.accessтомedicines.org)
- Medicines Patent Pool: [www.medicinespatentpool.org](http://www.medicinespatentpool.org)
- Media reports:
  - [http://www.reuters.com/article/us-health-hepatitis-medicine-idUSKCN0Y2AN](http://www.reuters.com/article/us-health-hepatitis-medicine-idUSKCN0Y2AN)
‘European systems for prices and reimbursement of medicines’

Lieven Annemans

Ghent University

July 2016

Article 25 - Universal Declaration of Human Rights

Everyone has the right to a standard of living adequate for the health and well-being of himself and of his family, including food, clothing, housing, medical care and necessary social services.
“Member states who invest in health policy today, contribute to the economic growth of tomorrow. Effective healthcare is one of the best possible investments we can do.”

Vytenis Andriukaitis
EU commissioner of health

key principles of healthcare
No blind investments

“We need to make available only those innovative technologies that offer an added value to patients and/or society at an acceptable cost, and fill unmet medical needs”

NOTE: ‘technology’ = devices, medicines, diagnostics, prevention programmes,…
- Report of the Belgian EU Presidency, adopted by the EU Council of Ministers of Health in Dec 2010
- European Commission – Investing in Health February 2013

What does HTA involve?

1. Synthesising health research findings about the efficacy, safety, and effectiveness of different health interventions
2. Evaluating the economic implications of the interventions
3. Analysing their cost effectiveness
4. Assessing social and ethical implications of the diffusion and use of health technologies
5. Assessing organisational implications of the diffusion and use of health technologies
6. Identifying best practices in health care
The Belgian approach for medicines

→ Class I: “if a medicine is claimed to bring an added value, and a higher price is claimed compared to the standard of care, then it is assessed according to the following criteria”

- Added therapeutic value
- Therapeutic need
- Impact on the Health care Budget
- Cost-effectiveness from health care perspective
Key problem in the EU

- Different P&R (HTA) criteria
- Different HTA processes
- Different Economic capacities
- Different social values

Difference in patient access

Towards changed roles & responsibilities in assessing innovative medicines

<table>
<thead>
<tr>
<th>Assessment criteria for innovative medicines</th>
<th>CURRENT</th>
<th>FUTURE</th>
</tr>
</thead>
<tbody>
<tr>
<td>Centralized EMA</td>
<td>HTA and competent bodies</td>
<td>‘Joint Initiative for Medicines’ (JIM)</td>
</tr>
<tr>
<td>Efficacy</td>
<td>✓</td>
<td>✓✓✓</td>
</tr>
<tr>
<td>Safety</td>
<td>✓</td>
<td>✓✓</td>
</tr>
<tr>
<td>Relative efficacy</td>
<td>✓</td>
<td>✓✓</td>
</tr>
<tr>
<td>Relative effectiveness</td>
<td>✓✓</td>
<td>✓</td>
</tr>
<tr>
<td>EU medical need</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>Local medical need</td>
<td>✓✓</td>
<td>✓</td>
</tr>
<tr>
<td>Ethical and social aspects</td>
<td>✓✓</td>
<td>✓</td>
</tr>
<tr>
<td>Cost-effectiveness</td>
<td>✓✓✓</td>
<td>✓</td>
</tr>
<tr>
<td>Budget impact</td>
<td>✓✓✓</td>
<td>✓</td>
</tr>
<tr>
<td>Organizational aspects</td>
<td>✓</td>
<td></td>
</tr>
</tbody>
</table>

Based on Annemans et al. 2011
Example: Added therapeutic value

- “1 per day versus 3 per day is/is not added value”
- “an effect on cholesterol does/does not guarantee an effect on cardiovascular disease”
- “a symptomatic effect is/is not enough to claim added value”
- The long term effect can be predicted/is unknown
- “the comparator is/is not relevant”
- “the clinical trial duration is OK/ too short”
- ...

→ Payers should avoid to jump to conclusions
→ Industry should make more clear what the added value is
→ Need for a common definition of added therapeutical value (“relative effectiveness”) per disease area?
Final thoughts

1. Investing in health
2. Innovate the way we deal with innovations
3. Towards
   - Joined assessment of relative effectiveness & added therapeutical value
   - Common principles in the assessment of cost-effectiveness and budget impact
   - Common principles for dealing with uncertainty
4. An unjust society leads to unjust health care
‘European systems for prices and reimbursement of medicines’

Lieven Annemans

Oher University

July 2016
Presentation by Mr Dirk Van Erps

Competition enforcement for pharmaceutical products

Dirk VAN ERPS
Head of Antitrust: Pharma and Health Services Unit
EP Workshop "EU options for improving access to medicines"
Brussels, 14 July 2016

DECLARATION
The views expressed are purely those of the writer and may not in any circumstances be regarded as stating an official position of the European Commission.

How Competition Takes Place

- Three stages in the life cycle of a pharmaceutical product:
  - Innovation → Market exclusivity → Loss of exclusivity
  - Research firms → Originator Pharmaceutical firms → Generic firms
  - Prescription drugs → Switch → OTC drugs

- Competition stemming from different players at each stage (case-by-case):
  - Innovation: pipeline products
  - Exclusivity: pipeline & marketed products
  - Loss of exclusivity: generic versions of the molecule
  - Over-the-counter: "consumer goods" (non-originator brands, private-label...)
How Competition Takes Place (cont'd)

- For example, generic competition ensures:
  - Incentive to further innovate
  - Less expensive medicines for patients and health budgets
    - approximately 25% cheaper at launch and 40% cheaper after 2 years
    - Health systems save up to 20% one year after launch and 25% after two years
  - Generics represent 50% of pharmaceutical consumption (80% of pills in UK vs 8% in Luxembourg)

2009 Report on Pharma Sector Inquiry

Sector Inquiry = Possibility for Commission to use investigative powers if indications that competition may be restricted or distorted

Sector Inquiry launched in 2008

2009 Report Conclusions:
- Intensify competition law scrutiny
- Support Community Patent and creation of unified litigation system
- Streamlining Marketing Authorisation Process
- Developing a pro-competitive environment for generic uptake
Pay-for-Delay Agreements

- Decisional Practice of the Commission suggests that such agreements do come under antitrust scrutiny (e.g. Lundbeck, Servier)

- **Patent Settlement Monitoring Exercise:**
  - Purpose is to better understand the use and evolution of patent settlements in the EU
  - Over the years the number of settlements is higher (76 in 2014) than at the time of the sector enquiry (+/- 30)
  - The proportion of *potentially problematic* patent settlements remains low at around 10%
  - Ongoing enforcement action and monitoring activity have not hindered companies from concluding settlements in general

Incentives to pay to delay

- Generic entry leads to erosion of prices and gains of market shares of previous originator’s monopoly. It also results in consumer savings

![Diagram showing impact of generic entry on market shares and consumer savings](image-url)
Incentives to pay to delay (cont'd.)

- To avoid the above effects of generic entry the originator by paying to delay continues to earn monopoly profits. In addition the potential consumer gains are shared between the originator and the generic(s)

→ The one who loses is the consumer!

Factual similarities

- Citalopram and perendopril were Lundbeck and Servier blockbuster medicines

- Basic patent for the molecules and original processes had expired. Thus, market was in principle open for generic competition.

- However, remaining process patents offered still limited protection.

- Several generic companies had made serious preparations to enter;
'By object' assessment:

- Potential competition between originator and generic companies

- Commitment of the generic company to limit its independent efforts to enter the market for the duration of the agreement

- Value transfers from the originator as a significant inducement which substantially reduced the incentives of the generic company to pursue its independent efforts to enter EU markets

By 'object' assessment – other factors

- That the value transfers took into consideration the turnover or profit expected by the generic in case of entry;

- That originator could not have obtained the same limitations on entry through enforcement of its process patents;

- That the agreement contained no commitment from originator to refrain from infringement proceedings if entry post-expiry of the agreement (Lundbeck) OR restrictions lasted throughout the entire period of the patent term (Servier)
By 'effect' analysis

- Market position of originator;
- Whether generic is potential competitor
- Content of the agreement (significant reverse payment changes the incentives of the generic party to accept the exclusive clauses of the agreement);
- Competition that would have existed absent the agreement (opposition procedure; patent challenges (non-infringement; invalidity); entry at risk; appreciable increase in likelihood of significant market power remaining uncontested for longer period of time and that consumers forego a significant reduction of prices that would ensue from timely and effective generic entry)
- Would competition be restricted in view of the existence of other relevant sources of competition to the originator
Role of EMA in authorising and supporting access to new medicines

Workshop
“EU options for improving access to medicines”
European Parliament, Brussels 14 July 2016

Presented by Enrica Alteri MD
Head Human Medicines Evaluation Division

Which medicines are approved through the centralised procedure?

- Human medicines for the treatment of HIV/AIDS, cancer, diabetes, neurodegenerative diseases, auto-immune, and other immune dysfunctions, and viral diseases
- Medicines derived from biotechnology processes, such as genetic engineering
- Advanced-therapy medicines, such as gene-therapy, somatic cell-therapy or tissue-engineered medicines
- Officially designated ‘orphan medicines’ (medicines used for rare human diseases)
**What is the benefit of the centralised procedure for EU citizens?**

- Medicines are authorised for all EU citizens at the same time.
- Centralised safety monitoring.
- EU-wide action in case of safety concerns.
- Product information available in all EU languages at the same time.

**EMA approvals in 2015**

<table>
<thead>
<tr>
<th>Positive Opinions</th>
<th>Positive Opinions</th>
<th>Negative Opinions</th>
<th>Withdrawn Applications</th>
</tr>
</thead>
<tbody>
<tr>
<td>93</td>
<td>39</td>
<td>4</td>
<td>5</td>
</tr>
</tbody>
</table>

**Noteworthy therapeutic innovations in 2015 included:**

**Cancer**
- Bincyto - directing the immune system towards cancer cells
- Farykyl - regulating the activity of genes
- Imlyvic - using genetically engineered virus to kill cancer cells
- Opdivo, Nivolumab BMS and Keytruda - increasing the capacity of the immune system

**Cardiovascular**
- Encresto - dual action to treat heart failure
- Repatha and Praluent - monoclonal antibodies to treat hypercholesterolemia

**Neurology**
- Wakix - action on histamine H3 receptors to treat narcolepsy

**Haematology**
- Praxbind - targeted neutralisation of the anticoagulant effect of Pradaxa
The typical long road of bringing medicines to patients

Development phase:

- Pharmaceutical and nonclinical (4–6 y)
- Phase I and II (2–4 y)
- Confirmatory phase III (2–5 y)
- Assessment and approval (1–2 y)
- Reimbursement and launch (0–2 y)

Access

Chance of reaching access for a product entering the development phase:
- 0.01-0.1% (0.1-1%)
- 5-10% (5-10%)
- 50-60% (50-60%)
- 75-90% (75-90%)

Regulatory provisions primarily targeting the time to access:
- Conditional MA
- Accelerated Assessment
- Compassionate Use ...

Regulatory provisions primarily targeting the risk of development failure:
- Scientific Advice
- Support to SMEs ...

The road to patient access

HTA

Drug candidates

- Does the drug do more good than harm in a defined group of patients?
- What are the health and cost consequences associated with this drug relative to other interventions in a defined group of patients?
- How does the drug perform relative to other interventions in this patient?
- Am I willing and able to pay for this treatment out-of-pocket?

Regulatory agency, Payer, Prescriber, Patient as payer, Market and patient access
EMA and HTA data needs – parallel EMA-HTA Scientific advice

<table>
<thead>
<tr>
<th>Regulators</th>
<th>HTA</th>
</tr>
</thead>
<tbody>
<tr>
<td>Benefit/Risk</td>
<td>Added value to recommend reimbursement</td>
</tr>
</tbody>
</table>

- Comparator: placebo, active comparator
- Clinical endpoints: Survival, quality of life
- Duration of the trial
- Patient population to be included premarketing / post marketing

- Single regulatory / HTA development plan feasible in at least 70% of cases
  - product development is more rational
  - products may reach patients earlier
  - patients will only participate to well-designed trials which will provide required evidence

EMA-HTA Parallel Scientific Advice
Experience so far – report published 31 March 2016

- 63 parallel EMA – HTA SA procedures with EU HTA bodies from UK, Italy, Germany, Sweden, France, Netherlands, Spain, Belgium, Austria, Poland, Norway, Hungary

- Broad range of indications:
  Lung cancer, Breast cancer, Pancreas cancer, Melanoma, Asthma, Diabetes, Heart Failure, Depression, Alzheimer’s, Migraine, Infections, Rare diseases, Myasthenia Gravis
PRIME (PRIority MEdicines) scheme - Goal & Scope

To foster the development of medicines with major public health interest.

- Reinforce scientific and regulatory advice
  - Foster and facilitate early interaction
  - Raise awareness of requirements earlier in development

- Optimise development for robust data generation
  - Focus efficient development
  - Promote generation of robust and high quality data

- Enable accelerated assessment
  - Facilitated by knowledge gained throughout development
  - Feedback of relevant SA aspects to CHMP

- Building on existing framework;
- Eligibility according to existing 'Accelerated Assessment criteria'

- PRIME is Patients’ needs driven: many patients with serious diseases have no or only unsatisfactory therapeutic options and should be able to benefit from promising medicines as early as possible.
- PRIME helps developers to optimise development plans for medicines that address an unmet medical need.
- PRIME fosters early dialogue with EMA to facilitate robust data collection and high quality marketing authorisation applications to speed up evaluation.
Workshop on EU Options for Improving Access to Medicines

PRIME Experience

- Biological
- Chemical
- ATMP
- Immunological
- Herbal
- Orphan
- Non-Orphan

Data excluding out of scope applications

First 6 products granted eligibility

- **CCX-168**
  - Treatment of patients with active ANCA-associated vasculitis (GPA and MPA)
  - Orphan

- **KTE-C19**
  - ATMP
  - Treatment of DLBCL, PMBCL, TFL
  - Orphan

- **CTL019**
  - ATMP
  - Treatment of paediatric patients with relapsed or refractory B cell acute lymphoblastic leukaemia
  - Orphan

- **Emapalumab**
  - Treatment of primary haemophagocytic lymphohistiocytosis (HLH)
  - Orphan

- **Adacumab**
  - Alzheimer’s disease

- **rVSVΔG-ZEBOV-GP, live attenuated**
  - Vaccination against Ebola (Zaire strain)
Key messages

- EMA approach to medicines authorisation and supervision at EU level has proved valuable and **benefits all European patients**

- Awareness of importance of steps that follow authorisation at national level:
  - Meet the needs of HTA as well as regulators has become essential
    - **parallel EMA-HTA scientific advice**

- Supporting development of medicines that benefit public health is a priority for EMA:
  - **PRIME scheme** and other early dialogue and support tools

Thank you for your attention

Further information

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Send a question via our website www.ema.europa.eu/contact

Follow us on @EMA_News
Access to medicines
A HTA perspective from Belgium

Raf Mertens, Frank Hulstaert
Belgian Healthcare Knowledge Centre, KCE

KCE, Belgian Health Care Knowledge Centre

- Advisory research institution, funded by federal public authorities
- Operational since 2004
- 50 researchers
  - medicine
  - economics
  - statistics, sociology, law, ..
- Studies (n>270)
  - Health technology assessment
  - Good clinical practice, guidelines
  - Health services research
Clinical development and HTA

**Clinical development**
- Exploratory trials
- Confirmatory trials (RCTs)
  - **internal validity**
  - **safety**
  - **efficacy**

**Health Technology Assessment**
- **external validity**
- **comparative effectiveness**
- **cost-effectiveness**
- **budget impact**

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External validity – comparative effectiveness

**THE LANCET**
Volume 380, Issue 9867, 11–17 January 2014,
Biomedical research: increasing value, reducing waste
*Malcolm R Macleod, Susan Nicoll, Ian Roberts, Ulrich Dinnes, Iain Chalmers, John P Ioannidis, Rostam Al-Shahbazi, An-Wen Chan, Paul Glasziou*

![Figure: Avoidable waste or inefficiency in biomedical research](www.kce.gov.be)
External validity – comparative effectiveness

- Go/NoGo decisions in pharma are public health decisions... but no public health decision makers are on board
  - Not really medical need-driven
  - Inadequate trial design and comparator, therapeutic benefit
  - High-priced new drugs

Adaptive pathways, Managed entry agreements, Access/coverage with(out) evidence generation

- **Before** market authorisation / coverage:
  - RCT (if required) is performed timely
  - Coverage can be obtained if efficacy is demonstrated

- **After** market authorisation / coverage:
  - RCT design is avoided, studies are delayed, safety risk
  - Coverage can be lost if efficacy is not confirmed
  - Difficult decisions, on poor data
Need for non-commercial trials

- Important research questions of interest to society will never be answered by industry (no commercial interest).
- Examples
  - Pragmatic comparative effectiveness trials
  - Repurposing of older drugs
  - Drugs in pediatrics and orphan diseases
  - Medical devices including diagnostics
  - Areas not owned by industry (surgical techniques, lifestyle, psychotherapy, population screening, ...)

Comparator

- best active
- active
- placebo
- none

Endpoints
- Quality of Life
- Survival

- pragmatic practice-oriented trial

- placebo-controlled trial

- narrow (efficacy)
- broad (effectiveness)
- Study population
Need for non-commercial trials

- Important research questions of interest to society will never be answered by industry (no commercial interest).
- Examples
  - Pragmatic comparative effectiveness
  - Repurposing of older drugs
  - Drugs in pediatrics and orphan diseases
  - Medical devices including diagnostics
  - Areas not owned by industry (surgical techniques, life style, psychotherapy, population screening, ...)

KCE Trials programme

- Well-designed, professionally run, publicly funded trials
- Immediately useful to patients, clinical practice (effectiveness), and policy or decision makers (efficiency).
- Extension of KCE HTA programme as at NIHR.
- National and international trials.
- Commissioned and researcher-led.
- Capacity building in the field
Cost-effectiveness – Budget impact

The Washington Post

Prescription drug prices jumped more than 10 percent in 2015, analysis finds

By Brady Dennis

The increase in new drug costs

Peter B. Bach, MD, MSKC
Sustainable Innovation

From the ethics of profit to the profit of ethics: sofosbuvir as an example of a medicine with an unsustainable cost, a dramatic challenge for health systems and a moral hazard for the industry.

Thousands of patients denied access due to exorbitant, unjustified prices... even in countries like Belgium.

Role of HTA?

- Price setting
  - + gamble on top!
  - threshold
  - Value-based pricing!

- Willingness to pay
- Moral obligation to pay

ICER Threshold (max € / QALY)

HTA

Budget Impact

Access or Rationing

Cost/QALY

Price of a life
High-priced new medicines

- On the agenda (OECD, WHO, UN, G7, G20,..)
- Council conclusions Dutch presidency

- KCE-ZIN scenario development project 2016
  - >30 Interviews + two workshops in Amsterdam
  - Investors, economists, payers, regulators, IP specialists, patient representatives, Mario Negri Institute, EORTC, Bill and Melinda Gates foundation, Cochrane Collaboration, policy makers, MIT, Harvard, MSK, pharmaceutical industry, ...
  - from Europe and North-America

---

Prices for new drugs have significantly increased and are putting a increasing burden on health care budgets in developed nations

The pricing logic is wrong

The drug development system is wrong

The values and principles are wrong

questions

answers
Building blocks in the scenarios

- Less waste by focus on public health needs
- Safety, efficacy + therapeutic added value
- Publicly funded regulatory system
- Preclinical + clinical data transparency
- PPP/public development
- Change/eliminate IP system
- Decouple R&D market from production/sales market
  - Reward; generic from day 1
- Compulsory licensing
- Drugs as a public good

Access to medicines
A HTA perspective from Belgium

...out of the box!
EU OPTIONS FOR IMPROVING ACCESS TO MEDICINES

European Parliament

Prices and Affordability

Fernando Lamata

Brussels 14 July 2016

Council Conclusions on strengthening the balance in the pharmaceutical systems in the EU and its MS, 17 June 2016

“…patients access to effective and affordable medicines is endangered by very high and unsustainable price levels, market withdrawal of products that are out-of-patent, or when new products are not introduced to national markets for business economic strategies”

“…several Member States expressed concerns that this [pharmaceutical] system may be imbalanced and that it may not always promote the best possible outcome for patients and society”
PRICES, DEPENDING ON THE METHOD OF PRICING

A. Without patent (no monopoly)
   - Market value + competition (near costs: generics): €160-€400

B. With patent protection (monopoly): over-pricing
   - Cost + over-price (manufacturing + R&D): €300-€600

-What the market will bear, without competition*
  
  Exorbitant starting price: €74,000
  --External Reference Pricing (ERP): €40,000
  --Differential Pricing + confidentiality (DP): €50,000 - €10,000
  --Health Technology Assessment (HTA): €50,000 - €30,000

*This is a contradiction. The justification for patent protection was to recover costs of R&D: Paying per “value” should imply free competition (without patent protection).
FINANCIALIZATION OF THE PHARMACEUTICAL SECTOR:
Strong pressure to raise prices

Patients / citizens → Pharma Industry: Too Big to Fall

Health Insurance/ Governments

Investment Fund → Financial markets

Financial flows → Products/services flows

PHARMACEUTICAL EU INDUSTRY:
(declared) NET MARGINS (2014)*

PHARMACEUTICAL INDUSTRY: 20% of sales

OTHER INDUSTRIES: 5% of sales

*Including in "costs" CEOs and executives compensation, excessive marketing, etc.
COMPENSATION\(^*\) OF THE 20 HIGHEST PAID CEOs OF THE PHARMACEUTICAL INDUSTRY (2013)

€ 13.2 MILLION - € 140 MILLION / YEAR

Average wages in EU: €4,000 - €40,000

\(^*\)(including salaries, bonus, option profits, etc.)

RESEARCH FUNDING THROUGH MONOPOLY OVERPRICING

Estimates for 2014 (data Efpia, 2016): Total Efpia pharmaceutical market value at exfactory price: €181Bn
Figures on the left, without overpricing of monopoly; figures on the right under monopoly

Inefficient spending: more than € 80 Bn
Unbalanced bargaining power by monopoly (patents, supplementary certificates, data exclusivity, etc.)

STRATEGIES AGAINST ABUSE OF MONOPOLY

- Price of brand product: (COE) + R&D + reasonable profit
- Price of Generic: (Cost without R&D) + set fee + reasonable profit
- Basic Price: (Cost without R&D + Reasonable profit)
- Independent R&D financing
- De-link R&D financing from prices (no patent)
- Compulsory Licence
- (2) Double
- (1) Negotiation
- (3) International Covenant

Strategy (1) short term; Strategy (2) short-medium term; Strategy (3) long term
Policy Department A: Economic and Scientific Policy

AUTHORISATION, PRICING, REIMBURSEMENT AND PURCHASE

1. New Medicine
   - Authorise Commercialisation
      - Efficacy; Safety Quality
         - Yes
         - No
      - Refuse Authorisation
   - No

2. Approve Price (under patent protection)
   - Yes
   - No

3. EU joint criteria
   - Cost; Manufacturing; R&D; Profit (5%); Reasonable initial sale price

4. Cost-Effectiveness;
   - Budget Impact;
   - Discounts/Rebates;
   - Volume agreements;
   - EU joint procurement;
   - Generics; Biosimilars;
   - etc.
   - Final price of purchase affordable

5. Compulsory License *
   - No
   - Yes

6. No reimburse; De-list

*Governments must gain negotiating power;
or / and change completely the system for financing R&D (monopolies)

PRICING AND AFFORDABILITY
One example: DNDi alternative

An alternative R&D strategy to deliver affordable treatments for hepatitis C patients, based on the cost of production

This regime (sofosbuvir + ravidasvir), if approved will be priced at less than $294 per treatment course (€261)

At this price, treatment will be affordable. It is a reasonable price. We can afford it.

SOME PROPOSALS
for controlling prices and increase affordability

Support cooperation between Member States and EU in order to:

- (under patent protection) Study and promote pricing based on (accurate) manufacturing and R&D costs, plus a reasonable profit

- Monitoring and investigation of potential cases of market abuse / excessive pricing (manufacturing and R&D costs vs prices charged by companies)

- Use Compulsory Licenses when patent abuse is evident and it is affecting patients and human rights. Review the model of financing research

- Develop public platforms for R & D: - priorities based on needs, - open and cooperative research, and - licensing for marketing at generic prices (reform IMI?)

- Strengthening the independence of Agencies and price regulators (EMA, HTA, etc.)
Workshop on EU Options for Improving Access to Medicines

Presentation by Mr Aquilino Alonso Miranda

Selection of meds to be dispensed in pharmacies and Andalusian Public Health Service hospitals
Aquilino Alonso, Andalusian Regional Health Minister

“EU options for improving access to medicines”

Spain: National Health System

- 100 % Public Funding
- Universal coverage (RD 16/2012)
- Access free of charge
- Integrated care
  - Primary Health Care (system’s core)
  - Hospital
  - Emergencies
- 17 autonomous (regional) health systems

“Mediterranean model”

Public planning & regulation of pharmacies to guarantee equity in access. (av: 1/2.200 hab)
Ley 14/2006, de 25 de abril, General de Sanidad
Ley 16/1997, de 25 de abril, sobre Regulación de Servicios de las Oficinas de Farmacia
Ley 19/2006, de 27 de julio, de Garantías y Uso Nacional de los Medicamentos y Productos Sanitarios (texto refundido: NUX. 1/2015, de 24 de julio)
Andalusian Public Healthcare System

- 87,597 km²
- 8,402,905 habitants (8,337,414 users)
- >1,513 Primary health care centers
- 48 Public Hospitals
- >95,000 Professionals
- 8,807 M€ Health Budget 2016 (2,346 M€ pharmaceutical expenditure 26.6%)

>3,800 pharmacies
(1 every 4, in rural areas)

Policies focused on Rights, Equity & Accessibility

Policies for a rational use of drugs
Sustainability, equity, quality & efficiency

- Prescription by active ingredient (93.37%). First region in the world (2001)
PUBLIC SELECTION OF PHARMACY-DISPENSED DRUGS. BACKGROUND

• Before this procedure started, pharmacies could choose the commercial presentation to be dispensed when a patient had a prescription by active ingredient. Pharmacies, then, could negotiate discounts.

• The Andalusian Public Health Administration decided to reverse these discounts in Public Health System savings, which means sustainability and benefits for the whole population.

• That’s why the public selection of these commercial presentations procedures began in 2012.

PUBLIC SELECTION OF PHARMACY-DISPENSED DRUGS. BACKGROUND

• WHEN THE PRESCRIPTION IS BY ACTIVE INGREDIENT
• TRANSPARENT AND OPEN TO FREE COMPETITION
• COMPANIES OFFER AN ECONOMIC DIRECT CONTRIBUTION TO ANDALUSIAN PUBLIC HEALTH SERVICE (CONTRIBUTION PER PACK OF THEIR PRODUCT DISPENSED BY PHARMACIES)
• AWARD BASED ON THE FINAL COST OF THE PRESCRIPTION OFFERED
• COMPANIES OBTAIN EXCLUSIVITY FOR THEIR PRODUCT FOR TWO YEARS
• COMPANIES ARE COMMITED TO THE PERMANENT SUPPLY OF THEIR PRODUCTS
PUBLIC SELECTION OF PHARMACY-DISPENSED DRUGS. RESULTS

Eight Calls (first: 2012; last: 2016)

505 drugs selected, 24 different labs

274 million euros savings for Andalusian Public Healthcare Service

“Efficient equivalent therapeutic alternatives”

Efficient Selection of “exclusive” drugs (under patent & without generics) with equivalent results in a certain health process, through:

- Use recommendations on the most efficient drugs in Hospital & Primary Health Care Reference Pharmacotherapy Guidelines (in collaboration with Scientific Societies)
- Specific goals related to this selection in Clinical & Management Agreements.
- Contract Public procedures for the selection of the best active ingredient to be dispensed in Hospital Pharmacy Services (except justified clinical reasons).
PUBLIC SELECTION OF DRUGS TO BE DISPENSED

Four basic ideas, from a strategic point of view:

- It takes time, it is impossible to achieve these results in a short term.
- The involvement of professionals is crucial. Quality oriented policies.
- It has to be aligned with the global health strategy, health results oriented.
- Political decision is needed, supported by citizens

Merci Grazie Thanks Dziękci Gracias Merci Takk
Presentation by Mr Pierre Meulien

The Innovative Medicines Initiative (IMI)

The Role of IMI in the Research, Development and Innovation in Medicines

Pierre Meullen
14.07.2016  EU options for improving access to medicines

What is the Innovative Medicines Initiative?

EU funding goes to:
- universities
- SMEs
- patient groups
etc...

IMI
> €5 bn

EFPIA companies
- receive no funding
- contribute to projects 'in kind'

EU
€2.5 bn

Partnership 2008 - 2024

Europe’s partnership for health

EFPIA
€2.5 bn
IMI 2 budget (2014 – 2024)

EU funding goes to:
- Universities
- SMEs
- Mid-sized companies
- Patient groups
- etc...

€1.638 bn

EFPIA companies receive no funding
contribute to projects ‘in kind’

€1.425 bn

Other €213 m

IMI 2 total budget €3.276 billion

An international, cross-sector community

970 academic teams

202 SME teams

552 EFPIA teams

31 patient crgs

108 other teams

Over 9 000 researchers working for:
- open collaboration
- improved R&D productivity
- innovative approaches to unmet medical needs
Why do we need IMI?
Because drug development is very...
- risky
- inefficient
- complex
- time consuming
- expensive

Because...
- Not enough science throughout development
- Clinical trial designs not always optimal
- Regulatory pathways not always optimised

All of this has a significant impact on the affordability and speed of access to innovation for patients
How is IMI addressing the challenges in drug development?

Through IMI’s projects we are trying to...

- put patients at the centre
- share risk (among public & private players)
- increase efficiency (by developing common tools)
- reduce duplication of effort (esp. at early stages)
- reduce timelines (by using a personalised medicine approach)
- integrate the latest science into drug development
- use data and knowledge management to work more effectively

We do this by creating a neutral platform where all involved in drug development – academics, industry, SMEs, patients, regulators, others – can engage in open collaboration on shared challenges.

“The IMI 1 projects reviewed were not designed to directly bring new medicines to market. Rather they will impact on new product development by acting on the medicines development process itself, usually in particular disease areas”

From the report of the IMI Socio-economic Impact Assessment Expert Group May 18th 2016. Available on IMI website
What is IMI delivering?

- 2,272 FTE jobs directly associated with IMI projects
- 169 SMEs
- 1,600+ scientific publications
- 65 clinical studies
- 20 patent applications
- 460+ biological marker candidates for better diagnosis & treatment
- 25+ new tools to facilitate drug development
- 13 spin-offs

Medicines safety – the challenge

- A major challenge in drug development is finding medicines that treat the disease but are not toxic to vital organs like the heart, liver, kidneys, etc...
- Too often, toxicity issues are only picked up late in drug development, when vast amounts of time and money have been spent on a drug.

IMI projects are developing simple tests to detect toxicity issues earlier in drug development.
Medicines safety – an IMI success

What eTOX did
Pharma data + Public data = One big database underlying multiple computer-based tools

Example: Will this be toxic to the heart?

Input = 2D structure of a possible drug

Output = possible effect on heart – ECG result!

Medicines development – the challenge

- We don’t know enough about the underlying causes of many diseases – we need this information to develop effective treatments!
- We don’t have the tools to study the underlying causes of many diseases

IMI projects are developing tools and establishing networks to study diseases and making new discoveries on the underlying causes of diseases.
Medicines development – an IMI success

**Autism...**
- affects 1 in 110
- results in difficulties in social interaction & communication, repetitive behaviours
- has a major impact on families & carers
- has no treatments

**Our EU-AIMS project has discovered...**
- new insights into underlying causes (genes etc.)
- that the brain changes associated with autism could be reversed
- gender differences in the brains of men and women with autism

**EU-AIMS**
- Patients involved
- Working with regulators
- Recognised globally as flagship project

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Medicines development – the challenge

- **High Throughput Screening (HTS)** = researchers screen large collections of chemical compounds in hunt for molecules that could be potential drugs or be used in drug development in other ways.
- **Pharmaceutical companies** have huge compound collections, but access to these is usually tightly restricted...
- **Public compound collections** exist, but are small and expertise is scattered across many institutions.

**Through its European Lead Factory project, IMI has created a state-of-the-art compound collection & screening centre that is delivering results for academics, SMEs & pharma**
Medicines development – an IMI success

Access to the European Lead Factory has fast-forwarded our drug discovery programme in the field of oncology by several years. – Hub Ova, Netherlands Cancer Institute

ELF support & its high quality compound library will allow Effecta Pharma to expand its drug discovery efforts for dengue and gives an important boost to tackling this viral disease. – Effecta Pharma, UK biotech company

✓ Leading role for SMEs
✓ Quality & diversity of compounds recognised
✓ Award-winning IP solution
✓ Happy users!

Stay in touch

- Visit our website www.imi.europa.eu
- Sign up to our newsletter bit.ly/IMINewsletter
- Follow us on Twitter @IMI_JU
- Join our LinkedIn group bit.ly/LinkedInIMI
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Presentation by Mr Maurice Galla

Strengthening the checks and balances in the EU pharmaceutical system

Outcome of the Dutch EU Presidency 2016

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Priorities Dutch EU Presidency

Key theme: Strengthening checks-and-balances in the pharmaceutical system

- Improve voluntary cooperation and exchange of information on pricing and reimbursement between Member States
- Support timely access to new, essential medicines by clarifying conditions and exit options
- Initiate debate on unintended effects of current incentives in EU pharmalegislation and their impact on innovation and costs
- Strategic debate on cooperation on future challenges and directions for pharmaceutical policy in the EU

Related key priorities: Medical Devices and in-vitro diagnostics and AMR
Meetings during NL Presidency

Political level:
- Informal meeting of Council of Health Ministers and formal EPSCO Council

Policy and technical level:
- Meetings of Directors responsible for pharmaceutical policy in Member States
- Conference on timely access to innovative and affordable medicines
- Competent Authorities for Pricing and Reimbursement (CAPR)
- Meeting of the European Network for Health Technology Assessment (EUnetHTA)

Industry:
- Round Table on pharmaceuticals between EU ministers and CEO’s industry

Why was this a priority?

The positives
- EU pharmaceutical system (public & private) is innovative; provides safe and effective medicines for the majority of its citizens
- ‘Special’ legislation for rare diseases and paediatrics have generated a variety of new pharmaceuticals that improve and save lives
- EU Member States can decide for themselves what pharmaceuticals they wish to pay for, adjusted to national needs
- EU has an important and relatively innovative pharmaceutical industry
- Public investments into R&D stimulates innovative and viable pharmaceutical products
Is pharmaceutical system out-of-balance?

The concerns

- Increasing mismatch between marketing authorisation topped up by incentives supporting innovation versus affordability of and access to final product
- Increasing focus of industry on development of (new) pharmaceuticals for smaller patient groups with high earning potential, not necessarily for unmet medical needs
- Essential medicines not available to increasing number of EU patients due to unaffordable high prices, withdrawals due to low revenues or (too) small markets
- Governments act individually, while counterparts act globally; Information asymmetry
- Examples of socially undesirable or irresponsible behaviour
- Public investment R&D often does not benefit public interest (tax payer pays twice)

Pharma system strongly interconnected

Concerns:
- Weak life MA and HMA
- Lack of data availability
- Significant benefit

HTA (valuation), pricing & reimbursement

Concerns:
- High prices, gouging
- Information shortcuts
- Globalisation of industry
- Negotiation imbalances
- Parallel imports
- Value-based pricing paradigm not accurate

EU Regulatory Framework
Market Authorisation
"Pharma system"
delicate balance

Concerns:
- Supply driven market
- Reverse incentives
- Underestimated market behavior (e.g. pay back delay)
- Medicines shortages
- Lack of competition
- Infringement of patents

Markets:
structure, business-models & competition
Informal Meeting of Health Ministers

- Several Member States agreed with analysis that imbalances exist in the pharmaceutical system and that action is needed.
- Support for voluntary cooperation on pricing and reimbursement, e.g. between economically similar countries.
- Support for assessment of the (un)intended effects of intellectual protection and additional incentives in the pharmasystem.

But:
- Voluntary cooperation should have clear added value; decisions on pricing & reimbursement remain MS prerogative.
- Tackling of unintended effects incentives should not discourage innovation.
- There should be a dialogue with Industry.

Council Conclusions
Strengthening the balance in the pharmaceutical systems in the EU and its Member States
Main aims council conclusions

- Political statement – recognition that imbalances exist in pharmaceutical system
- Address pharmaceutical system as a whole, with interlinkages and cross-silo/pillar (spill over) effects
- Aiming at ‘rebalancing’ the system to make it work as intended – legislation, innovation, incentives and national policies
- Initiate longer-term strategic cooperation to ensure consistency and continuity, ownership by all Member States

Actions for Member States

- Invest in voluntary and Member State driven cooperation on pricing and reimbursement
- This through joint activities, information sharing, joint negotiations with selected countries and cooperation on HTA, including through EUnetHTA
- Strategic policy reflection and exchange between Member States
- Development list of shared challenges and actions for 2017-2020, targeting the pharma system as a whole and its interrelations
- Collaboration across the system to ensure follow up actions
Actions for Member States & Commission

- Cooperate together and set clear and enforceable (pre-)conditions regarding the use of early access tools
- Further develop cooperation on Health Technology Assessment at EU level
- Improve and strengthen dialogue and cooperation in existing fora in the field of pharmaceuticals, while also assessing their relevance, functioning and added value
- Invest in essential R&D to address unmet medical needs and registries
- Also promote open access to data and ensure fair return on investment of successful publicly funded research

Actions for the Commission

- Streamline implementation orphan regulation; ascertain proper application rules, incentives and rewards; revise if necessary
- Create overview of EU pharma legislation in relation to IP related incentives and their intended purpose
- Analyse effects of these incentives on innovation, accessibility, availability and affordability of medicines, as well as price strategies of industry
- Analyse functioning of the EU pharma market in terms of transparency, market behaviour and competition and strengthen market oversight
- Recommend possible remedies in context of agenda 2017-2020
Thank you!

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Improving access to medicines in the EU

Andrzej Rys, Director
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Health Systems, Medical Products and innovation

European Commission
Directorate-General for
Health and Food Safety

The diagnostic - Common challenges

- Retail pharmaceutical spending account for 15% of health expenditure (OECD)
- Consumption of medicines increasing due to population ageing and new high costs products and technologies
- The proliferation of high costs medicines questions current pricing models – prices are disconnected from the health benefits they bring
- Financial sustainability of Health Systems under pressure
The debate on access to medicines

At national, EU and international level

- Questions about the proportionality of prices with health benefits
- Questions in terms of affordability and access to effective treatment for the patients
- Questions on the efficiency of pharmaceutical spending (value for money for health systems)

Council Conclusions: IT, LU, NL
EP own initiative report 2016
Stakeholders
WHO, UN, Observatory, USA etc.

Common solutions?
Balancing, value and sustainability

- Access to effective and affordable medicines for patients
- Value for money for patients and payers
- Sustainability of health systems
The Commission's role/actions

- Facilitate development and authorisation of innovative medicines for unmet medical needs
  → EMA / PRIME /ADAPTIVE PATHWAYS
- Identify ways to optimise the use of existing regulatory tools
  → STAMP
- Encourage with EMA parallel and coordinated work between innovators, HTA and regulators
  → EMA/EUnetHTA/ HTA Network
- Foster strengthen cooperation on HTA
  → reflection on going
- Address the request in the June 2016 Council Conclusions on incentives
  → Study 2017

The Commission's role/actions

Follow up to June 2016 Council Conclusions:

- Commitment to respond to Member States invitation to assess the impact of SPC, data protection and market exclusivity incentives from the health perspectives, and in particular on innovation, availability and accessibility

- Methodology and timeframe to be proposed by the Commission by the end of 2016
The Commission's role/actions

Initiatives on *orphan medicinal products*:

- A new Notice (to replace previous communication) to make orphan designation a more effective tool
  - to further encourage R&D for orphan medicines
  - to support that the best treatments receive a designation

- Technical revision of a Commission Regulation on similarity to reflect scientific developments for biological and advanced therapy medicinal products

The Commission's role/actions

*Paediatric Medicines*:

- Commission’s second report on the impact of the Paediatric Medicines Regulation to be published in 2017
- Analysis of public health case, as well as the economic impact of the Regulation
- Commission's study on a value of the rewards, the compliance cost for industry and the costs and value of the Regulation from the perspective of national health systems
The Commission's role

Encourage and support MS's voluntary cooperation

- Cost effective use of medicines as part of the EU Agenda for effective, accessible and resilient health systems
- Encouragement to rationalise pharmaceutical spending (Country specific recommendations in the European Semestre)
- Contributing to the debate (2016 study on pricing mechanisms)
- Providing a tool for exchange of transparent information on prices → EURIPID

Commission's role – HTA

What is HTA?

HTA is a powerful tool that assesses the added value (relative effectiveness) of a given health technology over and above existing ones.

Why is it important?

- Improves patients’ access to technologies with added value
- Allows Member States to take rational decisions how to best use health care budgets and thus contributes to accessible, sustainable, effective and resilient health systems
- Provides stimulus/incentive for the industry to innovate
Commissions role – HTA
EU Cooperation on HTA

**HTA Network**
- Policy and strategic cooperation
- Set up October 2013
- Art 15 Directive 2011/24
- MS representatives (mainly MoH)

**EUnetHTA Joint Action**
- Scientific and technical cooperation
  - JA 3 launched in March 2016
  - Co-funded by the Public Health Programme and MS
  - HTA doers (mainly HTA Agencies)

- These two levels work in synergy and complementary
- Involvement of stakeholders - both at strategic level and scientific level

Commission's role – HTA

HTA Network Reflection paper on synergy between regulatory and HTA issues – (END 2016)

- Address first the **pharmaceuticals sector**
- Life cycle approach:
  - premarketing phase
  - actual market entry
  - post-market launch phase

**EUnetHTA JA3 (2016–2020) : cooperation with EMA**
Commission's role - HTA

Reflection process

- EUnetHTA Joint Action to end in 2020
- Where do we want to be in 2020 when the Joint Action ends?
- Reflection process initiated to establish strengthened and sustainable EU cooperation on HTA after 2020
Role
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