Third Hearing of the Expert Panel on Effective Ways of Investing in Health

Residence Palace, Brussels, 25 October 2017

Scope

On request of the European Commission, the Expert Panel on Effective Ways of Investing in Health (EXPH) is producing three reports ('Opinions') on effective ways to invest in health. In this Hearing held in Brussels on 25 October 2017, the EXPH focused on the 'Opinion on innovative payment models for high-cost innovative medicines'. The aim was to shape and provide inputs for reflection on possible actions to be undertaken at national and European levels.

Opening of Hearing

Sylvain Giraud, Head of Unit, Performance of National Health Systems, DG SANTE, European Commission, welcomed all participants to this Hearing. His key points:

- Today's aim is to give all interested parties the opportunity to finalise the draft of the Opinion on innovative payment models for high-cost innovative medicines.
- This Opinion is driven by the Commission's interest in supporting MS towards more effective, accessible and resilient health systems. A further aim is to provide elements for the national level policy-making and EU-level cooperation on possible new ways of setting prices and paying for innovative medicines, so as to improve access to treatments that address evolving health needs and take into account the sustainability of health systems.
- The Opinion describes the variety of different pricing models that have been proposed, some of which are now operating already in Member States.
- The Opinion also provides key principles to address questions relating to benefits, timely access, and a framework for innovation and cost-effectiveness.
- The Opinion does not interfere with the allocation of national competences in the EU. Instead, it aims at providing all interested parties with useful input into the ways various public authorities and key stakeholders can work together in a continuous process from research to market access to pricing and reimbursement.
- Today's Hearing will enable participants to make statements and comments to the Panel members, based on their reading of the draft Opinion, as provided a few weeks ago.

Summary of Opinion and contribution to EU health policy development

Pedro Pita Barros, Professor of Economics and Vice-Rector at the Universidade Nova de Lisboa in Portugal, and Rapporteur of the Working Group, provided the Hearing with a short summary of the current draft of the Opinion. He concluded that no single payment model will fit all situations. His key points:

- Summarising the Terms of Reference, he said that the Panel was essentially searching for what is new in innovative payment models, especially in terms of a framework and what principles can be taken from other areas.
- Conclusion one: i) the current growth of innovative medicines cannot be continued indefinitely, hence the search for new ways to ensure innovation 'that matters', ii) that patients have access to innovation, and iii) that health systems are financially sustainable.
- Conclusion two: no single payment model will be optimal for all situations.
- Conclusion three: the EXPH proposes observing broad principles, based on discussions and documents.
- Eight proposed principles. These cover issues such as price and cost transparency, high-value innovations (including patents and market exclusivity), methodologies to measure the social value of pharmaceutical products, assessing the exercise of market power in price negotiations, better rewards for higher therapeutic value added, payment systems for services rather than products, non-linear payment systems, and creating dialogue platforms with all stakeholders.
- How the EXPH derived these eight principles.
- Summary of current practice of pricing new products, especially the use of Managed Entry Agreements (MEAs) for setting prices designed to control high prices and growing health expenditure. One starting point for the EXPH was overcoming the uncertainty about the value of a new product and setting its prices (which are two separate issues). Why high prices may result from a variety of reasons (high costs, high margins, higher margins for higher value products). Market power exercise is not assessed by current institutional mechanisms.
- Desirable end-term goal: keeping relative incentives for higher value products, without exerting today's financial pressure on health systems.
- Three key objectives to be achieved by payment model: i) innovation that matters, ii) giving patients access to innovation as soon as possible, and iii) ensuring that health systems are financially sustainable.
- Properties for payment models of innovative medicines: i) role of directing R&D (e.g. patents for decentralised R&D model, more centralised procedure for specific therapeutic gaps), ii) affordability to health systems/patients, iii) intergenerational effects (possible to have payment model with goal of all generations contributing to R&D?), iv) balance between objectives and instruments (e.g. inflexibility of linear price model; new ways to reward innovation rather than using price or patents), and v)

framing health system design options (e.g. a new payment model should include mechanisms to promote affordability, timely access and incentives for innovation with value).

- Governance is very important but a major challenge, especially in new payment models (e.g. need to involve all stakeholders, vital to have partnership decisions, important changes may be needed in legal and institutional settings of health systems).
- Instruments:
  - i) non-linear prices (e.g. combination of therapies akin to 'bundles' in other sectors, pricing analogous to that in other sectors like telecoms or water, price differentials across geographies and/or indications, prices reflecting economic opportunity costs);
  - ii) price transparency (e.g. knowing how prices are formed, knowing more about R&D, finding ways to disclose sensitive data on R&D/operations costs);
  - iii) from paying bills to paying services (e.g. new payment models based on outcomes, requiring different relationships between payers and suppliers, with governance challenges);
  - iv) innovation procurement initiatives;
  - v) incentive role of prices (e.g. finding ways to reduce prices without hurting the incentive to discover higher value products);
  - vi) searching for a new institutional design (e.g. hurdle role of HTA, negotiation still needed, calling on all instruments available including TRIPS, new payment models must address balance of power);
  - vii) real-world data and adaptive pathways (need for further information when using new payment models, real world data on a product is weaker than evidence from randomised control trials, political risk of delisting and costs risk of too quick introduction of low-value products);
  - viii) patents (will continue to be important in future but are not the only way to reward innovation, patents can be used differently by payers e.g. countries pooling to buy a patent for direct licensing);
  - ix) international cooperation (e.g. new payment models to reward innovation will require international coordination, ideally just for 'buyer clubs', more dialogue between all stakeholders, no existing payment model will dominate all scenarios, need to tailor payment models).
- Proposal of basic principles in design of new payment models (e.g. greater price and cost transparency, new rules to protect innovation – complementary to patents, new ways to fund R&D – especially in clear therapeutic gaps, adopting governance models to meet demands of new payment models, better methodologies to measure value/costs of pharmaceutical products, assessing exercise of market power in each price negotiation, setting better rewards for higher therapeutic value added, moving towards acquisition of service rather than products, exploring non-linear payment systems, creating dialogue platforms).

- Six proposals for action (based on suggested principles): relevant authorities in health systems asking for costs of R&D, marketing and production; international prizes for one selected area; checking existing payment models in each country against suggested principles; introducing a competition policy review ('comparison checklist') of high prices asked by companies; assessing value of new products of uncertain benefit; and strengthening bargaining power of health systems as buyers (joint negotiation procedures, mandatory licensing in extreme cases of public health risk).

Open discussion: stakeholders' views (round one)

Moderator **Walter Ricciardi** invited comments and questions on the draft Opinion and the preceding presentation. This led to contributions from seven members of the audience.

Ingmar de Gooijer, Director Public Policy & Reimbursement, myTomorrows, Netherlands.

The report is comprehensive, includes good insight and is valuable.

myTomorrows is rolling out a new drug access and development model, which it presented to the Dutch Parliament at a recent hearing on the high price of medicines. The model calls for a certain group of patients to be given specific medicines that are under development and to have them reimbursed. We use past/current legislation to ensure this is ethical. Through observational studies, we want to ensure that payers have more control, because we give them open access to the results, in combination with ongoing efficacy trials.

We also allow payers to negotiate drug prices before/after approval, which is based on efficacy and real-world data on effectiveness. myTomorrows believes this system enables the treatment of patients and to meet their needs. Price negotiations are a more rational model and a possible solution to one of the problems you discussed.

Professor Luis Abegão Pinto, Faculty of Medicine, Lisbon University and representing European Glaucoma Society (EGS).

We need a clear definition of the implementation of outcomes, which is not easy because clinical trials last three to five years. That period is too long to have an industry payment strategy. So we should not reinvent the wheel, but simply involve the existing partners. Use an advisory panel for negotiations.

Jan Van Lente, Director of EU Affairs, AOK-Bundesverband, Germany.

The Opinion and today's presentation of it were excellent.

I still have a problem with the concrete recommendations, especially those for the short term. Maybe we should differentiate between what we can do in the short and long terms. It feels unsatisfactory to me to say that we should apply those mechanisms to all products. The pricing system must be regular with some exceptions, e.g. for antibiotics. Why are there no recommendations for this? What can we now do in negotiations? You mentioned value and transparency of costs, but this does not ultimately help us to find prices. So it is important to strengthen the negotiating position of players, but only in the long term.

I was surprised that the EMA was not mentioned in today's paper. What is the EMA's role?

Henri De Ridder, Directeur Général du Service des soins de santé, National Institute for Health and Disability Insurance (RIZIV/INAMI/NIHDI), Belgium.

Congratulations on a very comprehensive oversight of the issues at stake.

The report's proposal to say that confidentiality should not really play between research companies and public authorities is an important part of the report, as confidentiality is a real hurdle to taking decisions.

Unmet medical needs might be one of guiding starting points for taking action in the field of access to medicines for example for developing new models. Hence the need to link with priority-setting in developing and bringing to market new medicines.

There is a concern about the capacity of payers to handle new approaches: this needs knowledge, people and resources.

Some of the concrete actions are already being tested, e.g. structured collaboration in the Beneluxa and Visegrad initiatives (sharing and pooling of capacities). However these tests are not yet sufficiently systematic.

Ioannis Natsis, Policy Coordinator, European Public Health Alliance (EPHA), Belgium.

Thanks for the excellent overview.

Managed Entry Agreements are not very innovative payments models now. See the August 2017 article ('How can pricing and reimbursement policies improve affordable access to medicines? Lessons learned from European countries.') http://www.lse.ac.uk/researchandexpertise/experts/profile.aspx?KeyValue=a.ferrario%40lse.ac.uk by Alessandra Ferrario from the London School of Economics and Political Science. Evidence

on the use of MEAs is now available from central and eastern European countries and it is sobering. MEAs seem to consolidate the shroud of secrecy. Does it make sense to have publicly funded health systems and compatibility with this extreme confidentiality? So we need a full review of MEAs, to see who really benefits from them.

As for the adaptive pathways mentioned in the Opinion, they are a paradigm shift. But there has been serious criticism of them and we must ensure they are not a Trojan horse for deregulation.

Mark Davis, Director, Government Affairs, EU & EMEA West, UK.

Health systems have traditionally paid for chronic therapy, where treatment is given over months/years and where benefits accrue over time. Yet industry is moving more towards delivering curative therapy, with a single payment for a treatment that accrues benefits over many years. Curative therapy potentially generates benefits from generation to generation. How do we deal with this challenge?

Alexander Roediger, Director of European Affairs, MSD Europe, and representing the Oncology Steering Committee, MSD.

MEAs are an agreement, so they offer good financial predictability. They are also a useful tool for long-term access to treatment, such as managing affordability and high prices in fields such as oncology. So there is no problem with confidentiality. But the new Opinion is unclear on whether or not you are in favour of MEAs.

Professor **Pedro Barros** responded to the above comments and questions.

In answer to the comments by Ingmar de Gooijer on prices and negotiation, he said that the Opinion takes a broad approach and it remains to be seen what will happen. However this area does not touch on how prices are formed.

In answer to the comment from Professor Luis Pinto on identifying gaps, Professor Barros said that outcomes must be clearly defined. As an economist, he did not feel qualified to offer an answer, although the Opinion does include the Panel's views. It takes a long time for a new drug to be clinically relevant, as happens in mental health treatment: this long period can affect the choice of payment strategy. Two issues are raised: i) how to structure payment in order to keep incentives for all parties during the process, and ii) how companies can manage the financial strain of waiting up to five years to receive payments. The latter is easier to solve, using financial mechanisms. Our Opinion recommends including more information on this point.

In answer to Jan Van Lente and the short- and long-term issues, Professor Barros said that it is true that the mechanisms are mainly for the long term. Finding a clear mechanism would be good, but it is hard because there is no mechanism to satisfy all the goals (which can themselves vary by period/product) with a limited set of instruments. It is important to be able to deal with a lack of information, due to the uncertainty/ complexity of health care. Moreover, payment systems are complex too, so it is hard to have one clear mechanism that is valid for all situations. As for the EMA, it is discussed in the governance part of the Opinion.

In answer to Henri De Ridder and the issue of having information on costs, Professor Barros referred to an estimate by Professor Andrew Hill, citing huge differences between the price of a drug in the US and UK. Having accurate information on the difference between the cost of getting the product and the price asked is powerful information, as it says a lot about the value split and can affect the pricing mechanism. As for confidentiality, he agreed that authorities should be able to handle payment models, but they need expertise to deliver these complex models. Regarding concrete actions being trialled across Europe, he said that the EXPH has already assessed those in order to contribute an integrated view of their main characteristics and what is being addressed and how.

In answer to the comments on MEAs from Ioannis Natsis and Alexander Roediger, Professor Barros said that these agreements are often viewed as either very negative or very positive. They are an instrument, so it is difficult to be sure they will deliver what people expect. MEAs are not a solution to all problems, as they are set up to address the uncertainty about the value of a product and they do not address the issue of margins set. So MEAs are useful if used correctly, but they must be properly designed. As for adaptive pathways, the Opinion shares the concerns of Mr De Ridder.

In answer to Mark Davis and the issue of curative therapy, Professor Barros said that the implication is that the benefit should include all the avoided problems in the future because there is a curative treatment now. While this increases the value one may want to give to the product, should a higher price be set as a result of this? Or should the price instead be spread over time – as a sort of 'intergenerational payment'? Professor Barros argued that having this benefit does not justify a higher price than is being charged already – although he agreed the issue deserves further debate.

Open discussion: stakeholders' views (round two)

Priv. Doz. **Claudia Wild**, Director, Ludwig Boltzmann Gesellschaft (LBI-HTA), Vienna, Austria.

I am a member of the EXPH from Austria and I am heavily involved in data.

Responding to Ingmar de Gooijer of myTomorrows and his proposal that some patients are willing to use unapproved drugs where their efficacy or safety have not yet been proven, Dr

Wild had a question: What is the difference between your proposal and just involving those patients in normal approved trials?

Regarding the role of the EMA, Dr Wild noted that the Opinion includes one proposal to raise the bar for the EMA, which is a public body but 85% funded by industry. So EMA should be told what society defines as a benefit. She added that in her expertise area, oncology, there is a need for more knowledge of the drugs that are approved (especially since 50% of them have no or only marginal benefits) rather than adaptive pathways.

Responding to Ioannis Natsis and the issue of performance analysis of MEAs, Dr Wild said the Panel can only have a standpoint if it becomes possible to find out if there is anything in the MEA package for the public payer or if the package is simply about collecting data and not really about societal benefits.

Ingmar de Gooijer, myTomorrows, thanked Dr Wild for her answers to his questions about which patients to include. He said that his company expected to meet soon with one of her colleagues to discuss the myTomorrows model for allowing the many patients (c. 95%) who have unmet medical needs and who want to enter medical trials because they have no other options or are excluded from treatment. He added that most European governments do have treatment rules enabling these patients to be included in the compassionate use agreement. However the problem is that they are not being paid for this, so companies do not get their drugs to these patients. As a result, myTomorrows is calling for a 'smart' way to get effectiveness data during their trials and to set price points based on this data. This should increase competition, which is key to lowering the price of drugs and will lead to price discussions.

Professor **Lieven Annemans**, Faculty of Medicine and Health Sciences, Pharmaco-Economics Center, Ghent University, Belgium.

Speaking as someone with 25 years' experience examining cost-effectiveness analysis of pharmaceuticals drugs, I believe this Opinion is very comprehensive and constructive and is a big step forward. However I have five recommendations:

i) We must distinguish between drugs with short- and long-term (five to 10 years) impacts, e.g. reward for discovery/procurement of patents. If you pay for discovery, somebody still has to pay for the further development of that discovery. So clinical trials are still needed and this should perhaps be done through public-private partnerships.

ii) Innovation that matters is a good idea, but we must be more explicit about short-term outcomes and the added value of an innovation. This will depend on the health burden to patients and the size of the therapeutic benefit. If we can

combine these two things, we can better measure the added value to society. Cooperation will help here: we need a common European assessment on cost-effectiveness and budgeting, rather than a decision by EU Member States. This can be done by setting clearer societal limitations on what we are willing to pay for extra gains in health.

iii) We must explore better mechanisms to discover societal value of drugs and what is the willingness to pay for extra units of health. We must also be clearer about how far the high budget impact can change society's willingness to pay. Hence the importance of finding a mechanism to assess affordability.

iv) For MEAs, we must distinguish between non-health outcomes-based agreements (which are sometimes purely discounts) and health outcomes-based agreements, which are the way forward.

v) The cost of R&D is not easy to assess, as the cost of failed development must also be taken into consideration. Rather than assessing product-specific R&D costs, we should look at the total R&D cost of a company and companies must be clearer about profit margins.

Ulla Närhi, Ministerial Advisor, Ministry of Social Affairs and Health, Finland.

Thanks for a comprehensive report and presentation.

You mentioned that we need good cooperation between Member States to ensure good results. But there are many differences between our countries and their medical treatments. So please provide concrete example of how we should act. Do we need a platform to achieve better results?

Olina Efthymiadou (MSc), Medical Technology Research Group, LSE Health, London School of Economics, United Kingdom.

Our debate today mainly revolved around MEAs. But as a researcher focused on monitoring agreements, I have an issue with the visibility of carrying out impact assessment studies on MEAs and the unavailability of useful data on using medicines and prices. We need to do meaningful assessment on how these variables have changed after implementation of MEAs. So my proposal is to include an MEA in the discussion, and to focus not only on the good and bad sides of MEAs but also to propose ways for collaborative action among Member States. This will ensure that MEAs are implemented in a more transparent and less secretive way.

Dr **Sarah Garner**, Coordinator, Innovation, Access and Use (IAU) Team, Essential Medicines & Health Products (EMP) Department, World Health Organization, Geneva, Switzerland.

This report contains some fundamental misunderstandings about evidence, e.g. regarding patients being excluded from trials and about survival. Although we can be critical of adaptive pathways and MEAs in today's regulatory system, I can see no alternative to them. What is important is when you have sufficient evidence.

Ancel.la Santos Quintano, Senior Policy Advisor, EU Projects, Health Action International (HAI), Netherlands.

Regarding value and society's willingness to pay for life-saving medicine, the sky is the limit. How do we solve the problem of today's high-priced medicines?

Simone Boselli, Public Affairs Director, European and International Advocacy, EURORDIS, Brussels, Belgium.

Thank you for the report. I have six points to make about it:

i) I would like clarification of the term 'neglected areas', as rare diseases have been in areas covered by the European Regulation.
ii) We welcome the recommendation about structured cooperation and would like to underline the reference to the Council conclusion about rare diseases under the Maltese Presidency.
iii) Some platforms exist (c. 15 Member States) and these should collaborate. EURORDIS has suggested European-level negotiations to set up prices for treating very rare diseases, e.g. through a mechanism of coordinated access.
iv) I highlight a pilot project involving several payers, patient groups and associations to discuss the type of data needed for better access.
v) We welcome joint procurement, which can drive additional innovation.
vi) We believe that MEAs are vital for reviewing uncertainties, e.g. marketing authorisation, especially for people with rare diseases and where the evidence is scarce and can be analysed through real-world data.

Dorothea Dalig, Stagiaire, Pharmaceutical Group of the EU (PGEU), and Vice-president of European Affairs, European Pharmaceutical Students' Association (EPSA).

Regarding the collection of real-life evidence, I note that some community pharmacies are already doing this. They are also saving historic evidence and patient reactions. So they are seeing real patients and not clinical trial patients, which is a major benefit.

Paul van Hoof, GlaxoSmithKline (GSK) and representing the European Federation of Pharmaceutical Industries and Associations (EFPIA) as Chair of the Committee on Access to Medicines.

Thanks for the report. My question to the Panel is whether you looked at everything on affordability. In the last 15 years, you can see stable or declining expenditure, due to competition among the innovative products and patent expires. Many biologicals are coming and lots of methods, yet many countries have caps on expenditure. So we need affordable applications as soon as possible.

Regarding patents and TRIPS, we should be concerned that there are now so many new and innovative medicines – especially for treating cancer and HIV – not that we haven't got enough.

Two of the round two comments were addressed by the moderator, Professor **Walter Ricciardi**, Università Cattolica del Sacro Cuore, Roma, Italy.

Regarding more structured cooperation between EU Member States, we have always urged the European Commission and Member States to use the immense amount of data we all have and to share the information with decision-makers. Our system is only possible if decisions are based on the interests of citizens/patients, in line with the evidence we have. Our Panel realised that this evidence is not used at all in most Member States when they take decisions. Europe is a paradigm of what is happening globally.

I was in Berlin last week with my colleague Professor Martin McKee to discuss Europe's role in the global health arena. Europe should be united, harmonised and use its many strengths. The European Commission should play a stronger role. If Europe focuses on trade and the economy, yet leaves health to Member States, we wrongly suggest to millions of people that health is not so important in Europe.

During my term as an independent member of this Panel, I was appointed in an institutional position in Italy, as President of the National Institute of Health (IANPHI). Yesterday we hosted 120 heads of national institutes from all over the world. We all face similar problems accessing the immense technology coming from public and private R&D.

In answer to my Finnish colleague, Ulla Närhi, we need more structured cooperation among Member States, with the European Commission (DG SANTE) included and pushing for that.

Regarding value-based health care delivery, we find that term rather ambiguous, as it is based mainly on the key work done by Professor Michael Porter in the United States. He was responsible for the famous concept of value in medicines, essentially based on outcomes and costs. Europe cannot afford that approach. We must be concerned with who will pay, such as taxpayers through social insurance, and focus on allocative value.

Professor **Pedro Barros** also responded to some of the round two comments and questions.

Our report should be clearer on the distinction between short- and long-term care, as well as on the meaning of innovation that matters.

Regarding the metrics of health per patient, we should probably say 'timely and safe access of patients'.

I agree about the views expressed on cost-effectiveness and impact, which is implicitly included when we discuss HTA.

Regarding non-health outcomes-based agreements and health outcomes-based agreements (MEAs), as an economist I like discounts: they are not always negative.

Regarding the further development of drugs, knowing the cost is still broadly relevant and our Opinion aims to highlight that.

Regarding specific proposals for action, maybe health competition authorities should be challenged when drug prices are too high. In the long term, we should get all Europe's ministers of health together, nominate a committee to quickly identify gaps in critical areas and get enough funds to reward companies for innovation, with a patent for anyone who wants to produce the selected drugs. Once the principles are defined for Europe, action can happen quickly, backed by political decisions.

Regarding value-based health care, it is not the same as value-based pricing. The latter gives a distorted view in the general economy and leads to high prices.

Regarding TRIPS, the concerns are not so much about having too many products on the market. People are more concerned about prices being too high for products entering the market: the problem is the prices asked, not the patents. TRIPS is a mechanism to influence negotiations and find a solution when there is no agreement. Our Opinion recommends using TRIPS when negotiations fail.

Lastly, regarding affordability/dynamics, I think it would be useful to tell companies that the prices for their products should be in the range of prices charged over the last 20 years. Yet the average increase per product is 10% or more per year. So we need a new structure and mechanism to think about long-term prices in the future.

Professor **Martin McKee**, London School of Hygiene and Tropical Medicine, London, United Kingdom, also responded to some of the round two comments and questions.

Regarding adaptive pathways/real-world data, our Panel is not ruling them out. We have no particular concerns about them and will happily look at any peer-reviewed published reports that challenge our interpretation of them.

Regarding the question as to whether national authorities and regulators have the ability to do all that is asked of them. This is a good point. I presume you would all agree we need to invest substantially in such bodies, so they can cooperate across Europe and in coordination with the European Commission. To serve the needs of European citizens, we need to redress the power balance.

Dr **Aleš Bourek**, Vice-President, European Society for Quality in Healthcare (ESQH), Czech Republic.

We need a wiki-like process – and the courage and time – to start collaboration in our field. The European Commission could guide us in that process, with a document as a starting point and then we could begin collaboration based on set rules, e.g. for pricing. This is an iterative process and could strengthen individual national associations and result in innovative models, with teams forming themselves and interacting.

Professor **Werner Brouwer**, Erasmus School of Health Policy & Management, Erasmus University Rotterdam, Netherlands, also responded to some of the round two comments and questions.

Regarding two points raised by Olina Efthymiadou, firstly it's very important to determine willingness to pay but it's hard to get specific pinpointed willingness to pay for quality. Secondly, we use a willingness to pay for quality in the traditional economic valuation sense, where we can compare benefits with costs. The problem here is the distinction between costs and prices. As long as we trust that prices reflect costs, people can make up their minds whether to pay for a particular drug. If there is uncertainty about this, then paying up to the threshold for willingness-to-pay means you give the entire surplus to the producer of the good. That is an undesirable effect of having an HTA on such a decision. As our report says, this is about more than reflecting on HTA in the traditional sense. Is there a fair division of the surplus, between the producer and the person getting that product?

Regarding budget impact, this is tricky because one could have a situation where one group of 100 people are judged by a different type of rule than are 10 groups of 10 people. We need to assess whether that is the correct way to look at such problems. So you are referring to 'non-marginal changes'. However, there are few interventions available that have a non-marginal impact on the value of health.

In response to Professor Brouwer, Professor **Lieven Annemans** suggested solving the problem of having a too high willingness-to-pay threshold (and giving all the surplus to the company) by setting this threshold lower.

As for budget impact, he said he raised this issue because some payers may think that affordability is the most important thing. This could lead to a very cost-effective drug not being accepted for its impact on the budget. Therefore budget impact should play a role, but only among other criteria including effectiveness, medical need and cost-effectiveness. I call this 'value-informed and affordable prices', where value plays a role but affordability must be used to mitigate the willingness to pay.

Open discussion: stakeholders' views (round three)

Jan Van Lente, Director of EU Affairs, AOK-Bundesverband.

Thanks for the clarification on EMA's role. My organisation favours raising the bar at EMA. But this needs discussion, because EMA will also give an opinion on the added benefit of some products which are not available on the national level of HTA bodies. We're not sure if this should be done by the EMA, due to a possible conflict of interest. In the context of the European Social Insurance Platform, we supported a different agency – one that is independent from EMA – to work on HTA.

The Opinion seems ambiguous about the need for transparency when pricing new/innovative medicines. Is this due to prices varying by Member States, parallel trade or price differentiation (e.g. per different indications, as happens now in Germany)? How can price differentiation be done and where is it functioning?

Dr **Sarah Garner**, Coordinator, Innovation, Access and Use (IAU) Team, Essential Medicines & Health Products (EMP) Department, World Health Organization.

Publication bias is a risk for peer review. The IMI consortia have released publications on adaptive pathways, but because we have been working on drugs going through the approval process, we can't publish on those drugs. Lots of that information is privileged for the agencies. There is also a problem for paired HTA agencies, with staff lacking time to publish.

Did the Panel consider other sources of evidence, not just the one on adaptive pathways? In your summary, you mention paying systems will evolve in direction of paying for acquisition of a service, not a product. Are you advocating that pharma companies go into health care delivery?

In the innovation process, it's important to distinguish between the molecular innovation and the process that the product has to go through with the company selecting the

indications to pursue for phase two and beyond trials. There should be a balance between scientific endeavours and commercial interests.

Is there any evidence for better rewards for higher therapeutic value added? Is it possible to do this using such mechanisms, since cost-effectiveness is one way you can charge high prices if a drug has higher added value. Is there any evidence this has changed the innovation efforts of companies or redirected their portfolios?

Professor **Martin McKee**, London School of Hygiene and Tropical Medicine, London, also responded to Dr Garner's comments and questions.

We obviously take on board a wide range of evidence. The evidence we looked at was based on rigorous work and available evidence.

I'm not sure it's right to say people are too busy to write peer-reviewed journals and that we should chat with them instead.

It's important for us that our discussion is transparent and open.

Silviu Popa, Senior Manager, EU Government Relations, Celgene, Belgium.

Could the Panel clarify its position on international reference pricing, which you imply is a good thing? Because the rationale for having confidential MEAs is also to avoid the undesirable effect of such pricing. But if we take a value-based approach, it doesn't make much sense: by referencing the price, you automatically import a perception of value which can differ from one country to another.

Professor **Luis Abegão Pinto**, Faculty of Medicine, Lisbon University and representing European Glaucoma Society (EGS).

As physicians, we aim to make cost-effective decisions based on knowing what value is – although we often disagree what that is. Many diseases are not about life and death when taking decisions, but are more a grey area. So where are the checks and balances on your value models, to ensure decisions are truly scientific or evidence-based? I believe it would be easier to use European societies' think-tanks to make such decisions based on value, then to have a one-on-one chat to a colleague about what is worthwhile/good.

Moderator, Professor **Walter Ricciardi**.

The value of our Panel is having a mix of skills and experience, with economists, clinicians, public health physicians, and experts in health systems and policies. We do use models, but

they must be evidence-based, as this drives the judgement of clinicians. So we are transparent and accountable.

Professor **Pedro Barros** responded to some of the round three comments and questions.

Differential pricing by region can be useful under certain conditions, as it's an average price. This can go down, because you can decrease volume in some places and increase it in others. It's important to have and to define a price scale for differential pricing. Ideally a product should be priced on the basis of value, but having insurance prevents clinicians and others from price effects. Hence the need for supportive clinical guidelines.

Parallel trade is a concern: it always limits the ability to have price differentiation. So price differentiation does not mean value-based pricing.

Providing an outcome is providing a service, it's not providing a product. You can get an outcome in different combinations and you can possibly use different combinations of procedures in different countries. Pharmaceutical companies will not replace ministers of health, but they will be close to providing a service rather than just a product.

Commercial interests certainly do drive innovation to select indications being pursued. Early dialogues can help steer cost-effectiveness, but this will not be the only instrument. So there is no silver bullet for innovation.

Regarding clarification of international reference pricing, we do need an anchor for the prices. Most of today's discussion – e.g. structured cooperation or cooperation between countries – has been about having a different anchor for prices. There is a risk that an international reference pricing system will be subverted by companies, public insurance or the government. So reactions/adjustments must be sorted out in the payment systems. I would not restrict MEAs to just discounts to avoid international reference pricing.

Closing: conclusions of the day and next steps

Moderator, Professor **Walter Ricciardi**.

Today's discussion has been very rich. It included many different viewpoints, which we hope will move us towards a solution. Health care is complex and there are no easy solutions. Europe faces some major problems in this field, which can't be fixed by money. To remain prosperous, Europe must be innovative and cannot continue to be so generous. With our demography, technology and the explosion of chronic diseases as well as the incredible

impact of new technology – such as drugs, medical devices, genetic testing and mobile apps – we must work together to find solutions.

In one EU-funded project, we're trying to design the future of health care according to trends. Of the four emerging scenarios, we only want one to happen.

Conclusions for this Hearing were provided by Professor **Jan De Maeseneer**, Chair of the EXPH, Ghent University, Ghent, Belgium.

1) Thanks to everyone for their contributions to today's Hearing and to our working group for tackling a complex issue in their comprehensive 60-page Opinion. This Hearing contributed a lot to the debate, underlining that health care is complex and there is no quick fix available for payment systems.
2) We showed today that we need to find the gaps (important social, health and curative needs) and to address them with appropriate strategies.
3) By crossing this debate with the one on the Opinion related to Primary Health Care performance Assessment, I would like to make a comment. In that Opinion, we stress the need for a bigger paradigm shift to goal-oriented care, looking at what matters to people and their life goals. Today we underlined an extra challenge: how to ensure our contributions to new medicines, etc. do contribute to what matters to people and their life goals. We know that people want to be able to function and enjoy social participation.
4) We need an open dialogue and communication with the public about those issues. That is hard today, because the debate is emotional. People are willing to pay for treatment of oncological problems, but for COPD-patients our solidarity is not always that organised. Another challenge is that debates related to long-term conditions, may be difficult, as people have a tendency to focus on the short term.
5) Lastly, we need to strengthen European collaboration at different levels in this field. I support the idea of a health agenda, which should play a key role in the European pillar of social rights. This debate can contribute greatly to that, since health is key to building social cohesion in the EU.

ENDS