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Market Access Newsletter

Editorial

by Pr. Mondher Toumi
EMAUD Chairman

Dear Colleagues & Friends,

In this second issue, we will highlight some of the latest developments in market access in Europe and especially in France, Germany and the UK. We are happy and honored to welcome two experts: Pr. Bruno Falissard, Professor at Paris University and Director of INSERM U669, who accepted our invitation to discuss current approaches of pricing of pharmaceuticals within the perspective of society and/or philosophical considerations; and Pr. Adrian Towse, Director of OHE, an authority in health policies who reviews value-based pricing in the UK.

Latest news in France where Noël Renaudin, President of CEPS (French Pricing Committee) was re-appointed and might not go to the end of his new mandate. Mr. Renaudin expressed publicly that 50 000€ will become in France the yearly selling price for reimbursed drugs. However value-based pricing is not questioned and will remain the foundation of the French pricing process. Value-based pricing could be defined in a simplistic way: better performing products deserve a higher price. This raises two questions: what are the transparent rules that will allow a fair selling price? If a new product reaches the market and is better than the existing one, already priced at the selling price the new one will displace the existing one that will face price cuts. The implementation of such a process might be complex and encounter legal hurdles. Such regulation will, on the one hand, increase uncertainty about return on investment as often comparative effectiveness between new products is known at a late development stage and, on the other hand, 50 000€ will become the industry target price for all new innovative products.

The German Bundestag approved the new health bill AMNOG (Arzneimittelmarkt - Neuordnungsgesetz) effective from January 2011. It will end free drug pricing in Germany and will apply to all new active ingredients and new indications. Manufacturers will have to submit a dossier to G-BA at the latest with market launch and, at the earliest, with an application for drug approval. Authorities will assess the medicine within three months. Drugs already on the market might be assessed as well.

G-BA will make a decision based on advice from IQWiG within three months. This might be: inclusion into a reference price group; if added benefit is recognised, negotiations of premium price between sickness funds and manufacturers can commence; and G-BA can then issue clinical guidance.

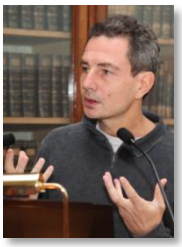
The Price has to be agreed within six months after the G-BA decision. In case no agreement is reached, arbitration will be applied and will be done by another committee (chairman plus sickness funds plus manufacturer). Then, if either party is unhappy with the arbitration process, an economic evaluation can be requested. Manufacturers will be advised to seek early advice from G-BA with respect to the first dossier submission and also prior to economic evaluation. It would have been *naïve* to believe that the free pricing in Germany would last longer. However the current reform looks reasonably balanced and could have easily been more challenging for the industry.

Value-Based Pricing (VBP) in the UK, evolution or revolution? It seems that the foundations and principles remain very similar. UK is facing two significant changes: moving from an ex-post VBP to an ex-ante VBP and integrating the "society perspective" in the valuation. The health minister has confirmed NICE will be "moved" from its current central role in health technology assessment to make way for value-based pricing of medicines [...even though we will rely on NICE's advice, we will move onto our own value-based pricing system [VBP]." From a conceptual perspective this is just an evolution, however for the industry this is a true revolution. UK organizations will have to reengineer their way to address market access in the UK. It will mark the end of high level British prices that are used for international reference pricing, although the pound devaluation already eroded what has become a myth. Pricing launch sequences will have to be reconsidered in the light of those changes.

The coming decade will face major changes for market access making this topic even more central to company's success. No continent will escape the emergence of more stringent market access regulation. On the top of major reforms at national level, the increasing pressure at the regional and even local level will force the industry to adapt their organizational structure and decision processes to take up these new challenges.

The resistance to changes might be the hurdle within giant organizations, while the lack of appropriate resources might be the hurdle of smaller organizations. *"It's not the strongest of the species that survives, or the most intelligent, but the one most responsive to change"*. Charles Darwin

Phenomenology and Habermas' public sphere as necessary complements to the utilitarian perspective for pricing of pharmaceuticals



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Obviously, pricing of pharmaceuticals relies on scientific considerations. Indeed, randomized controlled trials, cost-effectiveness studies and more generally evidence-based medicine are currently the basis for price determination of medications in most countries. Pricing of pharmaceuticals is also a political question.

First, because pricing is clearly an "affair of state" (one of the definitions of the adjective *politic*). Second, because pricing is related to moral issues: when one takes the decision that orphan drugs or drugs for the end of life could be less cost-effective than other drugs, this decision is indeed based on moral considerations. The pricing process of pharmaceuticals is thus basically a complex process and our job is to make this process more rational and efficient. The objectives of this paper are: 1/ to show that the pricing process of pharmaceutical relies on formal and informal considerations; 2/ that both formal and informal issues need to be considered rationally and that 3/ phenomenology could help to rationalize the informal aspect of the job.

Two basic ways for an access to knowledge

If we return thousands of years ago to the beginning of occidental philosophy, it was already considered that there are two basic ways to access knowledge: a direct one and an indirect one. Indeed in Plato's works rationality and even mathematics were very important. It is even said that Plato engraved at the door of his Academy "Let no one ignorant of geometry enter". On the other side Plato's work is brimming with myth which were as many pieces of poetry designated to propose another way to access knowledge, a more intuitive one. The opposition and interdependence of intuition and logic is thus ancient and its trace can be found in many classical antagonisms: induction versus deduction, diagram versus demonstration, informal knowledge versus formal knowledge and even on a neurobiological perspective, with the right brain opposed to the left brain.

The pricing of pharmaceuticals: two basic approaches

When you look at the pricing process of pharmaceuticals, in many countries, both these aspects of access to knowledge are present. Indeed, it is said that the NICE (National Institute for Health and Clinical Excellence), in England, use a formal criterion of cost per QALY (Quality Adjusted Life Year) to determine if a medication should be reimbursed or not. On the other hand, it is said that in France there is a transparency committee where pricing or effectiveness is decided informally by a vote. Of course, this is only a simplistic description of reality.

Expert Viewpoint

The decisions of the NICE are not only based on a formal procedure; and the decisions of the transparency committee are indeed based in part on evidence based medicine. There is in fact a real continuum between a purely formal approach and a purely informal one.

Rationality in formal and informal processes

It is basically easy to make the formal part of a decision more rational. ICH guidelines, good practices for cost-utility analyses or for mixed treatment comparisons: all participate to an enhancement of the level of rationality of the formal aspects of the evaluation process. But what about the informal aspects? Is it possible or is it even conceivable to make them more rational?

In the pricing process of pharmaceuticals emotions are not absent. In an "Appraisal Committee" of the NICE, for example, members of associations of patients are of course emotionally involved with medications, but it can be also the case of experts who can be patients in turn or whose parents or husband/wife can be patients. Infants, the elderly and the disabled are categories of human beings which are not neutral when it is time to discuss about their needs for care. And of course everybody knows that money and power are a source of emotions, and money and power are not unfamiliar with the world of pharmaceuticals.

Phenomenology: the objective study of subjective topics

Phenomenology is a rather old fashion philosophical movement founded in the early twentieth century by a German philosopher, Edmund Husserl. The objective was to find conditions for an objective study of judgment - perceptions and emotions. It is an anti reductionist approach. Phenomenologists try to grasp the global picture of the problem instead of cutting the problem into little pieces. The practice of phenomenology is a combination of disciplines and detachment to suspend or bracket theoretical explanations and second-hand information while determining one's "naive" experience of the matter" [1]. This can appear a bit awkward. In fact, many clinicians have a phenomenological practice, especially in psychiatry. In front of a complex situation, which may involve intense emotions in both patient and physician, the psychiatrist can stop him/herself during a few seconds and says inwardly "well at the moment I am doing my job. I am in front of this patient whose characteristics are likely to bias my judgment and I have to be apart a little bit to try to get rid of all my *a-priori* to be as clean and lucid as possible with my decisions". This is typically a phenomenological approach.

Habermas' public sphere

We live in democracies and since pricing or reimbursement of pharmaceuticals is a political decision, it has to rely on democratic bases. Some often argue in favor of the cost per QALY approach that since it is based on revealed preference obtained from the general population it is basically democratic.

This is not clear at all. Democracy is neither the tyranny of the majority (or of the mean in the case of QALYs) [2] nor the tyranny of a leader (elected or not) [3]. Jürgen Habermas, a German philosopher, has developed an interesting conceptualization of democracy [3] where the notion of deliberation is central. The democratic process consists of three steps: 1/ deliberation at the level of the citizen (inside the family, at the pub, etc.), 2/ deliberation in lobbies, political parties, specialized circles and 3/ the final decision taken by an elected parliament or president.

At the moment, politicians and payers hide themselves behind cost-utility assessment, evidence-based medicine, in order to avoid their responsibility, unaware of the reality: that a pricing process is a political decision which is based in part on moral considerations. A lot of progress has been made in the formal technical scientific part of the process. However, a huge progress has to be made regarding the human part of the decision. Therefore, the pricing process should rely on data, on evidence based medicine, on cost-utility evaluations but also on a public deliberation not limited to associations of patients. Citizens are needed in the process and they should deliberate in public meetings about the problem of health priorities. This is a first level of deliberation and we have seen that other levels of deliberation are necessary. They cannot correspond to elected people because the determination of a price needs a high level of expertise. The second level of deliberation should be restricted to a circle of lucid, enlightened people who know not only the technical aspects of the problem (e.g. methodological considerations) but also all the parameters that could interfere between their decision, their vote and themselves. Therefore, a rational objective approach to subjective judgment should be developed.

References

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[2] Tocqueville, *Democracy in America* (Harvey Mansfield and Delba Winthrop, trans., ed.; Chicago: University of Chicago Press, 2000)

[3] Habermas, Jürgen (German(1962)English Translation 1989), *The Structural Transformation of the Public Sphere: An Inquiry into a Category of Bourgeois Society*, Thomas Burger, Cambridge Massachusetts: The MIT Press, p. 30, ISBN 0-262-58108-6

Value Based Pricing in the UK: an update

Origins and Basics of UK VBP

The UK Government published its consultation document in December on Value-Based Pricing entitled "A new value-based approach to the pricing of branded medicines" (Department of Health, 2010). Introducing VBP was a commitment of the UK Coalition Government and formed part of the recent White Paper on health reform "Equity and excellence: Liberating the NHS." VBP was originally proposed by the UK Office of Fair Trading (OFT) in a 2007 Report.

Expert Viewpoint

The OFT proposed VBP to replace the profit control in the current Pharmaceutical Prices Regulation Scheme (PPRS). The consultation gives a first indication of how that could be achieved when the current PPRS ends in three years time in December 2013.

The DH consultation sets out a "QALY-plus" approach for drug pricing for branded drugs from 1 January 2014. The starting point for price evaluation would be a cost per Quality Adjusted Life Year (QALY) calculation. This would assume a "basic threshold" based on the opportunity cost of alternative uses of money within the NHS, currently estimated to be £20,000 - £30,000 per annum.

The "plus" bit of the pricing calculation would take into account the drug's ability to deliver one or more of the following: tackling disease with a greater "burden of illness"; demonstrating "greater therapeutic innovation"; and demonstrating "wider societal benefits". All of these would increase the price above the "basic" level. NICE will do the basic cost-per-QALY part with "expert groups" doing the "plus" element of the pricing. The paper leaves open who might bring it all together, and how that might happen.

Price flexibility and handling uncertainty

The 2009 PPRS introduced Patient Access Schemes (PAS) and Flexible Pricing to better enable drug prices to reflect value, in a response to the OFT 2007 Report. The consultation states that PAS would cease to exist under the new VBP system. A review by Towse (2010) indicated that most PAS have been financial – lowering effective NHS transaction prices below list prices. Their disappearance could create problems. Companies are not usually prepared to compromise on the NHS list price because of international reference pricing. The paper is silent on what options would replace this role of PAS.

The other reason for PAS is to handle uncertainty about the value of a drug at launch. Outcome-based schemes including risk-sharing can in principle be used. The document (clause 5.8) says that "one approach might be to set a price that is supported by the evidence available at launch, but to allow prices to be adjusted as better evidence becomes available." This implies that the flexible pricing arrangements, allowed in the PPRS, will continue. NICE approved in December 2010 its first outcomes-based PAS, for GSK's pazopanib (Votrient). The price includes "a possible future rebate linked to the outcome of the head to head COMPARZ trial" (NICE, 2010).

Flexible pricing was also introduced in the 2009 PPRS in recognition that different indications have different value – again a principle set out in the OFT Report.

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Clause 4.19 of the consultation keeps this – talking about giving different prices for different indications. Implementing this may be challenging.

In another clause (5.9) on price assessment timeliness, the paper says "companies could make drugs available at a contingent price, which will subsequently be adjusted to reflect evidence of effectiveness". This means that a company can set a price until the VBP review takes place. The paper does not set any timelines on how long price assessments will take, but the NICE process could act as a precedent.

NICE mandate

Currently drugs recommended in NICE Technology Appraisals must be funded by the local NHS. This "NICE mandate" will remain in place during a "short term" interim period until the long term outcomes framework is in place that will drive good practice in the NHS; in clause 5.12 the paper says the government "will continue to ensure that the NHS in England funds drugs that have been positively appraised by NICE".

The NICE mandate is very important. The big danger with VBP is that all the effort goes into determining price and no effort goes into achieving the efficient volume corresponding to the price. If value-based revenue is zero, patients have no access and companies get no return on innovation.

Replacing the PPRS?

The DH consultation proposes using VBP for new drugs (although questioning whether to exclude orphan drugs). Drugs launched *before* 2014 will therefore be covered by arrangements alongside VBP requiring "a successor scheme to the PPRS." The OFT 2007 Report proposed introducing VBP within a PPRS recognising the importance of a stable national agreement between government and industry. The DH consultation talks of moving away from the 5 year PPRS renegotiation to "a more stable framework" offering "companies greater certainty for making long term investment decisions." The implication is that future change will occur with less frequency than every 5 years. This seems rather optimistic.

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•NICE (2010). Final Appraisal Determination. Pazopanib for the first-line treatment of advanced renal cell carcinoma. Issue date: December 2010.

•Towse A (2010). British Journal of Clinical Pharmacology. Value based pricing, research and development, and patient access schemes. Will the United Kingdom get it right or wrong? Volume 70, Issue 3, pages 360–366, September 2010

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Event: the 2nd Annual Market Access Day

The 2nd Annual Market Access Day took place on 3 December in Paris. For the second time the Ecole du Val de Grâce welcomed over a hundred of participants who were willing to hear more about "fair price" with experts from North America and Europe answering the question: "Pricing of pharmaceuticals: is there a universal foundation to set a fair price?". Speakers evaluated the performance of the pricing systems currently in place and explored what could be the answer to setting a 'fair price' for pharmaceuticals. Many felt that although all reforms aim at aligning value and price, affordability has become a key element of market access. It is therefore difficult to draw specific lessons applicable to all markets and to supply a single answer to define what fair pricing is; however, many countries are currently trying to integrate explicit affordability limits in their pricing practices.

Programme available upon request.

EMAUD Alumni

EMAUD has created its own Alumni group on Linkedin for students and contributors. The objective is to discuss latest topics on pricing and market access, promote activities and events. Students, join us, connect with us!

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