INFORM

Payers and Reimbursement

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Pricing and Reimbursement of Medicines: Who Will Pay for Innovation? (Part 1)

ALBERTO GRIGNOLO PhD

Special Section Editor

Back in the 20th century, life was easy. The hard part was obtaining regulatory approval for a new medicine. After that, financially sound markets, vast pharmaceutical sales organizations detailing to individual doctors and relatively permissive advertising and promotional practices helped sponsors achieve very handsome pricing and commercial success for their new medicines.

What a difference a millennium milestone makes. The landscape for the commercial success of medicines has changed radically in the majority of world markets. Economic pressures on both governmental and private payers have dramatically increased the burden of proof on sponsors to demonstrate the value of innovation for both patients and for those who pay for medicines. The outcome all too often today is that what is touted as "innovation" fails to impress those who are in a position to pay for it, and does not qualify for reimbursement. The race for "reimbursable innovation" is on, and it is of Olympic proportions.

In this 2-part series, the *Global Forum* has asked a number of experts to describe the current and future environment for pricing and reimbursement of medicines around the world. In this issue we examine this topic in Europe, the United States and Japan. Readers will immediately recognize these as the "ICH regions," the cradles of regulatory harmonization. But, whereas ICH has brought these regions closer together in terms of regulatory convergence, they remain far apart in the area of medicines reimbursement – ranging from nearly full coverage in Japan to sadly inadequate coverage in the United States, to deep fragmentation and heterogeneity of Health Technology Assessment (HTA) practices in Europe. The contrast is stark, but, there is a common denominator nonetheless: the stronger-than-ever preference of payers everywhere to pay for demonstrated therapeutic value. Read on.

Speaking of convergence: read about the emerging dialogues among sponsors, regulators and payers in the European Union – *during* drug development. Is it the shape of global things to come?

In part 2 (October issue) we will go farther afield in Asia and Latin America. Please join us. ●

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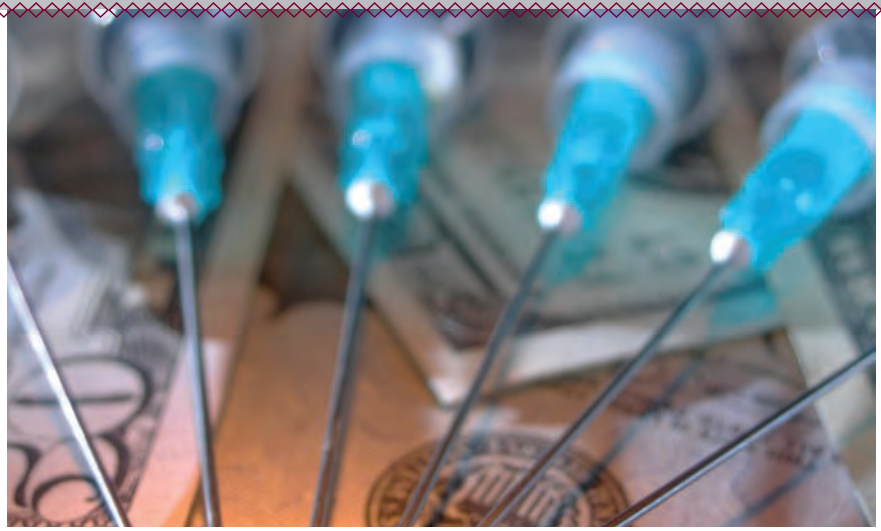
The Payer/Reimbursement Environment in the United States: Current and Future

CHARLES A. STEVENS, JD, MBA

Vice President & General Manager, Commercialization Strategy PAREXEL Consulting



The payer, reimbursement and patient access landscape in the United States has consistently taken a backseat in the product development process to the regulatory approval requirements of the Food and Drug Administration (FDA). For many years, sponsors were most worried about and invested the most resources in developing strategies and tactics that drove a product through the approval process. FDA approval was often akin to crossing the finish line in a marathon; today it is simply an important mile marker along the marathon route. Today's market has shown that a new regulator, the "payer regulator," is playing a very important role in determining if a product will be commercially successful post approval. Unlike the FDA, which is a single body that outlines the process and requirements for product approval at the outset, payer bodies in the United States are very diffuse and up until now have not offered actionable guidance on what type of product will meet standards for reimbursement approval.

The healthcare reimbursement and payment system in the United States is not only very complex, but is also very large from a monetary perspective. In total, healthcare spending in the United States constituted 18.2% of GDP (Gross Domestic Product).[1] The payment system is comprised of a variety of both public and private legal entities. Healthcare in the United States is paid for by hundreds of separate legal entities who offer thousands of different insurance coverage products. Government and public sector payers are now the largest payer of healthcare in the country, accounting for upwards of 50%

of all expenditures. This spending comes from programs such as: Medicare, Medicaid, Children's Health Insurance Program, Bureau of Indian Affairs, Veterans Health Administration, TRICARE and the Public Health Service. Despite the large number of government programs, the greatest number of Americans receive insurance from private healthcare payers. In 2010, that number was estimated to be at 195.6 million people, with 49.9 million or 16.3% of the population having no health insurance.[2]

The market is beginning to understand that there is more to product development and development success other than just obtaining FDA approval. Success is being measured by the value that a new product or technology offers to the market. Value as defined by benefits in clinical outcomes and safety vis-a-vis cost. This was driven home by Janet Woodcock, MD, in her address at DIA's 2010 Annual Conference when she said, "And so, even in the United States, we can approve drugs and we'll get them on the market, but for them to be successful, they are going to need to have demonstrated value … If you can show tremendous value for your drug, even in a small patient population, the payers are going to pay for it, because there's a population out there that will truly benefit. And I believe that's the future."[3]

Dr. Woodcock's statement speaks to the value that the market in general, but specifically payers in the United States, are looking to see from new products receiving FDA approval. Public and private payers have been working to limit drug costs while encouraging use of products that provide patients with the best outcomes. To date these have been demonstrated by payers developing coverage policies that require the use of generic and established branded products before granting access to newer expensive agents whose limited data sets come from controlled registration trials. Medicare, through the Medicare Modernization Act (MMA) of 2003, established a drug payment rate for Part B drugs under its Average Selling Price (ASP) system.[4] This "de facto" price control system requires sponsors to report the average selling price of their products quarterly, and then uses that ASP as the basis for reimbursement six months in the future. This lag time in updating reimbursement rates limits the ability of sponsors to take significant price increases.

The future of the payment and reimbursement system in the United States very much lies with passage of the Affordable Care Act (ACA). Provisions within the ACA created the Patient-Centered Outcomes Research Institute (PCORI) and have begun to focus all market stakeholders on understanding Comparative Effectiveness Research (CER) and how "real world" data can be used by payers to make coverage decisions.[5] There are several private payers who have begun to analyze their own internal claims data to determine which therapies provide the best value as measured in outcomes and costs to their patients. One example is WellPoint's policy change regarding the use of Zyflo, Singulair and Accolate.

WellPoint analyzed claims data of 55,000 patients and sent quality of life surveys to 800 patients to better understand how their physicians were prescribing oral asthma drugs in the "real world" community clinical setting. WellPoint determined that its prior authorization process for denying use of several products first line, due to a lack of FDA indication, was actually adverse to patient outcomes. The analysis of these data led WellPoint to remove its prior authorization and prescribing restriction for Zyflo, Singulair and Accolate. The benefit of this policy change was realized by all three major stakeholders: patients, providers and the payer.

Patients were able to experience better outcomes without having to be previously treated with therapies that were not successful, and providers were able to more easily treat patients without having the added burden of having treatment decisions denied by the payer. For its part, WellPoint realized cost savings as a result of patients being treated early with the most appropriate therapy. They also saved resources that were being committed to managing a prior authorization process.

There are also examples of an emerging and growing collaboration between government regulators and payers. Collaboration between FDA and the Centers for Medicare and Medicaid Services (CMS) has been occurring for some time on an informal basis, but now it is being formalized. The FDA and CMS signed a Memorandum of Understanding (MOU) in the summer of 2010 to share information and then announced plans in September to establish a process for the parallel review of medical products.[6,7] The two

agencies have separate and unique missions as mandated by Congress; nonetheless, FDA and CMS interaction is certain to increase to ensure that both agencies have the data and expertise needed to make appropriate decisions.

All of these market changes require sponsors to rethink their product development planning to include stakeholders who will be key drivers of product success post regulatory approval. The ACA, payer coverage decisions and increasing patient costs for treatment through co-payments and co-insurance make patients, their providers and caregivers important development stakeholders.

As the figure above demonstrates, sponsors must actively embrace new market concepts and bring many key stakeholders together to support product success post approval. Principles of personalized medicine and CER also will drive trial designs, discussions with regulators and shape post approval observational research.

The future state of the payer and reimbursement environment in the United States will continue to evolve, especially now that the Supreme Court has upheld the ACA.[8] This evolution will be driven largely by the need to reduce overall cost of care, requiring sponsors to establish product development teams that include clinical, regulatory and commercial expertise. Doing so will provide the best opportunity to develop products that offer a level of value that will be embraced by "payer regulators" as the reimbursement landscape in the United States continues to change. ●

References/citations from this article are available upon request.

Charles A. Stevens *is responsible for leading the development of strategic and tactical commercialization and market access solutions designed to support the commercial success of biopharmaceutical products, devices and diagnostics. He has over 20 years of bio and pharma industry experience, and has been responsible for strategic reimbursement, pricing, public and private payer strategy development, product distribution/ channel management and pharmacoeconomics for both commercialized and non-commercialized products in specialty markets such as Hematology, Oncology and HIV/AIDS. Prior to joining PAREXEL he worked for Abbott Laboratories, Genetics Institute (Wyeth Oncology), CellTherapeutics and Cephalon Oncology.*

Trends in Pharmaceutical Pricing, Reimbursement and Market Access in Europe

DR. FLORIAN TURK

COMMAND HEALTH ECONOMIES IN "POST-PHARMERGING" MARKETS

The EU healthcare systems are mostly in a post-pharmerging phase and defined by some common system characteristics: i) price has been suspended as allocation mechanism and been replaced by bureaucratic-corporatistic rationing framing the purchasing processes, ii) mounting healthcare expenditure due to growing demand (as an income effect of increasing welfare), malfunctions in the systems (e.g., moral hazard) and inefficiencies in health care delivery as well as iii) enormous and increasing economic pressure.[1] In consequence the systems are trying to maintain coverage and quality while managing margins and expenses through efficiency gains, alternative funding strategies and further fine-regulations of health service and goods allocation mechanisms.

Although there are wide variations in how the pharmaceutical industry is regulated by individual market states, the need for negotiating price and reimbursement terms following marketing approval – replacing market price as allocation mechanisms - means there is a considerable amount of time before patients are allowed access to new drugs (delays of more than nine months

between market authorization and market access are common in many markets). Supply side and demand cost-containment measures are in place throughout the region. Supply side efforts include price controls (such as general price freezes, general price cuts, individual price cuts, reference pricing), reduced reimbursement rates, negative lists of unreimbursable drugs (with erectile dysfunction, baldness and smoking cessation products being the most common), parallel trade, and (unofficially) the delays created by pricing and reimbursement negotiations. Demand side efforts include promotion of rational use guidelines, generic substitution, prescribing budgets, limits on sales and pharmaceutical spending, profit ceilings, promotional expenditure restrictions, prescribing budgets and targets, prescribing guidelines and restrictions, prescribing audits, prior authorization, step therapy protocols, incentives for generic prescribing, generics substitution, reimbursement reductions, and patient out-of-pocket payments increased copayments and restricting who can prescribe certain expensive products.

EXEMPLARY SYSTEM TRANSFORMATIONS SIGNALING THE LIKELY DIRECTION FOR PHARMACEUTICAL MARKET ACCESS IN THE EUROPEAN UNION

After a first wave of micro-regulations trying to manage pervasive effects created by the multiple demand- and supply-side regulations, most markets in the EU are currently facing a second wave of reforms with the aim to improve efficiency, quality, and allocation efficiency while helping to manage margins and expenses. The future outlook and different facets of the further system transformation will be described by three main trends observed across jurisdictions: i) value-based pricing, ii) more exacting evaluation of new drugs and iii) harmonization across jurisdictions.

i.Value-based pricing: Two main changes can be seen as a move towards value-based pricing in EU countries: the efficiency frontier approach in Germany as well as the proposed introduction of value-based pricing in the United Kingdom. In Germany, the efficiency frontier approach as laid out in the Institute for Quality and Efficiency in Health Care (IQWiG) methods[2] was understood as value-based pricing. The efficiency frontier can be extrapolated to assess whether additional benefits of drugs are worth additional costs compared with current therapies in a therapeutic field. According to the law before 1 January 2011, this would yield a maximum reimbursable price for a new drug or a drug of relevance, as defined by the National Association of SHI Funds. This stands in the tradition of Australia[3] and Canada[4]. In a recent consultation paper from the UK Department of Health, a new value-based approach to the pricing of branded medicines was proposed and will probably replace the Pharmaceutical Price Regulation Scheme (PPRS) after 2013.[5] A system known as “therapeutic innovation and improvement weighting” will be developed to reward therapeutic advances. In the event that the data available at the time of launch are insufficient to permit a full assessment of a new drug’s value, the VBP system would likely set a contingent or interim price and then conduct a full value assessment as soon as possible. The NHS will be required to reimburse therapies already approved by NICE and treatments included in the VBP system.

ii.More exacting evaluation of new drugs: This trend is reflected by the introduction of an early benefit assessment in Germany similar to that operated by the Scottish Medicines Consortium[6]: In December 2010, the German government passed the Pharmaceutical Market Restructuring Act, more commonly known as Pharmaceutical Restructuring Act, legislation designed to overhaul the German pricing and reimbursement system.[7] For all new drugs (and all subsequent extensions of indications for these drugs) launched in Germany after December 31, 2010, manufacturers will be required to submit a value dossier at the time of launch of new chemical entities and within four weeks of launch in the case of new indications for an established drug. Based on the value dossier, the Federal Joint Committee of Physicians, Dentists, Hospitals, and Health Insurance Funds will conduct a rapid assessment of the benefit of new drugs. This assessment will seek to determine the patients and diseases for which a drug offers additional benefit and what

competing therapies (if any) are available in Germany. All new drugs will be assessed for their additional benefit in comparison with appropriate therapeutic alternatives. As required in other countries such as France or Canada, the extent of additional benefit of the new drug will be categorized: (i) remarkable additional benefit; (ii) considerable additional benefit; (iii) minor additional benefit; (iv) additional benefit not quantifiable; (v) no evidence of additional benefit; and (vi) less benefit than the comparator. The dossier must include all studies conducted by the manufacturer and information on the approved indication, benefit, additional benefit in comparison with appropriate therapeutic alternatives, costs of treatment, number of patients and patient groups experiencing a therapeutically relevant additional benefit, and any special requirements to ensure appropriate use of the new drug and the predefined comparator. A similar trend can be observed in France: The French government's recent announcement of its intention to restrict reimbursement to drugs that can be shown to be at least as good as alternatives that are already reimbursed might signal the reintroduction of the former ASMR VI rating that was applied to drugs excluded from reimbursement because they were deemed inferior to their respective comparators as well as the French government's plan to call on the EU to adopt more-exacting standards for marketing authorization, based on comparative data relative to a reference product (if one exists).

iii.Harmonization: European health ministers first considered initiatives to harmonize HTA activities in the EU as long ago as 1991. In recent years, steps have been undertaken to increase the availability and transparency of health technology pricing and reimbursement information across countries. For instance, the Pharmaceutical Pricing and Reimbursement Information project commissioned by the European Commission provided a framework for comparability of pharmaceutical pricing and reimbursement data and policies, developed a core set of indicators, and conducted comparative analyses based on country profiles[8]. The Organisation for Economic Co-operation and Development carried out a similar project that generated a taxonomy of pharmaceutical pricing and reimbursement policies, and analyzed the cross-national impact of these policies[9]. Both initiatives also focused on the role which health technology assessment (HTA) and economic evaluation play in informing pricing and reimbursement policies.

The EU has influence on market access in the form of the EU Transparency Directive (89/105/EEC), which defines rules with regard to the procedures and timing of drug pricing in member states. The Transparency Directive is intended to facilitate the free movement of medicines within the EU. The directive states that pharmaceutical pricing procedures

must be completed within 90 days, with the possibility of a further 90 days for reimbursement decision making.

On March 28, 2011, the European Commission (EC) launched a public consultation with regard to the pricing and reimbursement of medicines in the EU. All interested parties were invited to share their views on the EU Transparency Directive which has not been amended since 1989 despite substantial changes in the pharmaceutical market. It's main driver has been the expectation that transparent pricing and reimbursement procedures contribute to maintaining a dynamic pharmaceutical market and can help diminish the strain on public health budgets. The consultation should help the Commission determine how to best update the existing rules to reduce pharmaceutical prices, guarantee transparent national procedures and facilitate a broader and timely access to medicines. The consultation addressed the following key issues: i) the consistency of current transparency rules with the development of increasingly innovative medicines, ii) the evolution of cost-cutting measures at national level, iii) delays in pricing and reimbursement procedures and the potential of developing an EU system of penalties for such delays, iv) the role of the European Court of Justice's case law in interpreting current rules and v) the opportunity to expand the scope of the Transparency Directive to include medical devices.

Furthermore, the EC has reportedly given serious thought to the creation of a "Euro-NICE," a pan-European HTA agency. In February 2010, the European Medicines Agency and the European network for Health Technology Assessment (EUnetHTA) launched a new initiative to identify ways for European HTA organizations to make better use of European Public Assessment Reports (EPARs) in assessing the relative effectiveness of medicines. The European Medicines Agency publishes EPARs for all drugs that are authorized through the agency's centralized procedure. EPARs provide summaries of the committee's scientific conclusions but omit information of a commercially confidential nature. The European collaboration around HTA is set to continue, given that HTA was listed as a high priority in the European Union Health Programme 2008–2013.

CONCLUSION

Pharmaceutical pricing, reimbursement and market access will undoubtedly become more challenging for drug manufacturers in the years to come. Given the enormous and increasing economic pressures on all European countries, postmarketing cost-containment measures will be a focus of governments' strategies, but politicians will also seek to strengthen prelaunch controls by stricter regulation of prices, stronger linkages between value and prices and more exacting reimbursement decision. To what extent EU initiatives will act as counterpoint to observed decentralization with complex set of regional and local cost containment measures (e.g., Italy, Spain) will become more apparent with the outcome of the European consultation on drug pricing and reimbursement. ●

References/citations from this article are available upon request.

Dr. Florian Turk

Dr. Florian Turk is Executive Director and Global Head Health Economics & Outcomes Research, Critical Care Business Franchise, Global Product Strategy & Commercialization at Novartis Pharma AG in Basel, Switzerland.

Prior to his current role Dr. Florian Turk held different roles in Market Access, Pricing, and Health Economics & Outcomes Research at Schwarz Pharma AG, Novartis Pharmaceutical Cooperation, US, and Novartis Pharma AG. Dr. Florian Turk graduated with a Master of Economics and received his Ph.D. in Economics in 2003.