Conference Proceedings

Summary of the International Pharmacoeconomics Forum: Focus on Oncology

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BACKGROUND

The "International Pharmacoeconomics Forum" took place in Riyadh, Saudi Arabia on November 15 and 16, 2019, to explore the topic of "Focus on Oncology."

The purpose of this paper was to register the Forum's main topics and conclusions, in order to build a foundation for future work in the area. Accordingly, it offers a general view on the event and focus on actionable ideas. As a result, individual participants' position on topics are not disclosed and the talks are summarized according to the main topic or conclusion.

The Forum discussed ways to improve patients access to quality-care medications at an affordable cost, by means of learning about (1) the cost implications, (2) the burden of diseases, and (3) the processes of managing health technologies assessment (HTA). In addition, the audience was introduced to several methods for cost-effectiveness analysis. In the end, the Forum supported the view that the way forward was to bring together all stakeholders and collected suggestions on how to reduce the rising trend of the costs of care and drugs, while maintaining a high-quality standard of care and providing good outcomes for patients. The event developed with these discussions in an environment suitable to sharing good practices, valuing different experiences, and introducing itself as a landmark for the audience.

ON THE IMPORTANCE OF THE FORUM TOPIC

The stakeholders and experts gathered at the "International Pharmacoeconomics Forum," acknowledge the use of HTA tools and the fact that a pharmacoeconomic rationale will further benefit the decision-making process in healthcare. As a reference, the European Network for Health Technology Assessment defines HTA as "a multidisciplinary process that summarizes information about the medical, social, economic, and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner."[1]

HTA has several uses, including being an integrated part of the decision-making process that sets the price or reimbursement of health technologies, an input into market access decisions, and establishing guidance on the appropriate use of products. As a result, HTA is performed by a variety of countries, systems, organizations, and others, such as the National Institute for Health and Care Excellence, the Scottish Medicine Consortium, the Portuguese Pharmacy and Drug Institute, or the Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen. These techniques are especially useful for prioritizing decisions on resource allocation. Furthermore, HTA methodologies can assess a wide range of factors. The most common techniques concentrate on combining clinical outcomes and their cost-effectiveness by using metrics, such as the incremental cost effectiveness ratio or quality-of-life measures.

Performing HTA involves always involves the identification, measurement, valuation, and comparison of the costs and consequences of the alternatives under analysis.[2] However, there are a variety of technical approaches. As a result, it becomes clear that further discussion on the use of these methodologies is of paramount importance, both in day to day decisions and at a strategic level by providers, payers, or regulators. The Forum expects to offer a contribution for this topic.

ORGANIZATION OF THE FORUM

The Forum was built around three technical modules, a discussion on international experiences in a question and answer format and two more modules discussing research experiences and practices, open to participation from the community.

The first module focused on the hot topic of the costs of medication. In order to build on impact and to provide useful insights for controlling these costs, the

Forum discussed concepts, such as cost effectiveness models. In addition, compared value of care frameworks, namely those from the American Society of Clinical oncology (ASCO)[3] and the European Society for Medical Oncology (ESMO).[4] The Forum also debated the burden of the disease of cancer, the role of medications at the treatments and on their impact on costs, and how these costs are controlled in practice in the Kingdom of Saudi Arabia (KSA) as well as other geographies.

As a clear sequence, at the second module, the Forum discussed the role of the pharmaceutical companies to keep the costs under control and the use of generics or biosimilars in a safe and efficient way.

The third module evolved from the systemic discussion to hospital level, by questioning the role of hospital leadership and the role of the hospital pharmacy in those processes. The Forum also explored the contribution of the therapeutics committees or the physicians to achieve this goal, without compromising high-quality outcomes for patients.

The discussion of the international experiences would follow, in a question and answer format in order to promote live discussion of interesting topics and some debate among the experts in the Forum.

Finally, two very innovative modulus on "case studies and practical examples" and "sharing experiences" allowed for the participation of several authors, around a total of 12 cases. Together with four communications at the satellite symposia, the Forum achieved a good balance between descriptive, normative presentations, and real-world, local, and international examples of the application of the health economics methodologies, reinforcing their role at supporting better decisions for the stakeholders in the healthcare value chain.

DISCUSSION

A Key Role for HTA

Module one established the importance of financial sustainability for cancer care, while sustaining the best possible outcomes for patients. In this sense, the discussion combined a high scientific level of discussion on HTA methodologies, with the pragmatic approach of analysis real-world elements of value in healthcare delivery, in a more holistic perspective.

The burden of cancer will increase between 2% and 5% year to year. However, the extra spending will likely bear positive outcomes for the foreseeable future. This has to do with the extra complexity of healthcare, not just the role of drugs or a single stakeholder, so all resources and all stakeholders need to be involved in the solution. Pharmacoeconomic assessments are key for the solution and they should be incorporated in the decision-making process. In fact, given the sensitivity and impact of the decisions around cancer, one can only expect these will be deeply rooted on rigorous analysis and research.

The basic rationale for the operation of the Ministry of Health and related bodies of decision and products committees, would ideally encompass first the budget impact models. Then, in order to value clinical outcomes, would evolve into regular cost effectiveness analysis. Once those data on quality and patient-reported outcomes have been collected, just like the international trends in the area of HTA suggest, cost effectiveness and cost utility models could be computed and offer a better insight to support decision on priorities and pricing levels to negotiate with pharma companies. Finally, for rare diseases, where traditional HTA might not offer a sufficient negotiation tool, then combining real-world evidence with multicriteria decision-making would be a possible technical supporting tool.

In cancer and rare diseases, value is uncertain because of the high prices. This is an opportunity for HTA to support access-related decisions with sound methodologies. But we need to have patient level real-world data (RWD), to perform real world studies and other locally adapted HTA models. The results of publications and industry sponsored studies are also good strategies to increase awareness and impact of HTA on people's lives.

Recently, the ASCO proposed a new 'framework' to assess the value of different therapeutic options in oncology that uses, among others, the following arguments[3]: not only is the cost of cancer treatment increasing but it has one of the highest growth rates compared with other diseases; in some cases, the adoption of newer and more expensive diagnostic and therapeutic interventions cannot be properly supported by medical evidence, thus increasing costs without improving the results; there are studies that show patients want to receive financial information about the different treatment options, together with information on effectiveness and toxicity; patients tend to overestimate the benefits of a treatment, which sometimes increases overall survival by only weeks or months, or may even have no impact; and there are doubts among oncologists about whether and how treatment costs should affect their recommendations.

In the absence of tools for measuring the magnitude of the clinical benefit of anticancer drugs, the ESMO developed a Clinical Benefit Scale. This tool reliably develops a ranking of the magnitudes of clinically significant benefits that can be predictable from a new anti-cancer treatment.[4] With this instrument, the ESMO takes "an important first step to the critical public policy issue of value in cancer care, helping to frame the appropriate use of limited public and personal resources to deliver cost-effective and affordable cancer care."[4]

Both the ESMO and ASCO proposed their value assessment frameworks. Nevertheless, the frameworks are not equal and there are numerous examples where treatments seem to be valued differently.[5] Consequentially, these frameworks should be used with caution, but they are good tools to promote discussion and to

highlight potential impact areas or value streams in novel technologies in healthcare.[6] Finally, the Forum discussed how they can easily be combined with traditional HTA frameworks and methodologies, offering further light into these gray areas.

The Role of Institutional Stakeholders

On module two, discussion ensued on different approaches regarding medication cost controls and the roles of stakeholders. First, biosimilars and generics are a tool here, and conclusions were best summarized on the expression "if you trust your regulator, you trust your biosimilar drug." This is even more clear on generics where one should expect the same result from all brands. The main fact is adopting technologies that are cheaper may save resources to other diseases and help to reduce inequalities in healthcare by improving access, compliance, and adoption.

The regulatory process for the approval of drugs can also be further improved. One direction is to use the "totality of evidence," such as the European Medicines Agency, the Food and Drug Administration, and other agencies. Of course, for biosimilars an abbreviated evaluation would suffice and increase speed to market, as more savings can be captured from an early launch of a quality biosimilar. The way forward to save money without losing on quality would therefore incorporate a specific approval process for biosimilars, audit and control capabilities on the regulators side, and thinking as normal to have generic and biosimilars reaching a high share of the market; in the United States it is now above 80%. This way we can benefit from the drugs and give access to more patients.

Cost effectiveness and other HTA tools were referenced by the Forum as a driver through the road to sustainability. Additionally, managed entry agreements, clinical trials, or simply efficient regulatory pathways were mentioned as critical to allow patients to have an early access to the therapies and to increased clinical benefit. In order to achieve these goals, there are shared roles, for example, on the side of the pharma industry, that should adopt a position of flexibility to make agreements easier and sooner.

Even for central governmental agencies, or for other payers, that currently choose on basis of tenders or reference pricing mechanisms, there is a complement role for HTA, which is not only to better define the price that the technology is worth, but also to support discussion on priorities and relative assessments of treatments. What is more, hospitals, providers, or purchasing organizations can also construct their assessments with HTA and introduce a second tier of negotiation with suppliers before the drug gets to be prescribed by physicians.

The Role of the Healthcare Professions

Oncologists would play a key role because they have an integrated vision on the patient. The same applies to pharmacists and other medical specialties. Decisions in oncology are a shared responsibility because the pressure comes from several directions, including the societal dynamics (e.g., ageing), the burden of the diseases that have clear social determinants (e.g., diabetes or lung cancer), and also from the price of resources (e.g., salaries, drugs, devices, etc.). People are living more years, which is good, but they will require more resources to remain healthy through this period. Other sources of pressure include evolving the delivery model, pursuing simultaneously excellent health outcomes, patients' and stakeholders' satisfaction, and keeping the lowest costs to provide the right care.

Industry (pharma and devices) also has a role. Some examples were reviewed, such as to invest in research and development and push forward (real) innovation, and to promote education for professionals and for the public, for example, by cooperating with public health campaigns. Likewise, the industry will help to sustain healthcare budgets by accepting to negotiate prices, to sign managed entry agreements, and in some cases by accepting that resources being limited, but because there are patients who can benefit from drugs, those budget caps are a pragmatic short cut to access.

The notion of value in health was also discussed, in line with the state of the art by Porter et al.,[7] such as a special taskforce from ISPOR.[8] The big question is how to implement value-based solutions at all levels, namely, how to achieve systemic impact drawing from individual initiatives. This way, shifting to value-based reimbursement models for pharmaceuticals or devices, it is an opportunity to create more sustainability in healthcare delivery and in drug purchasing. As of the present days there are already some examples of value-based purchasing and risk-sharing agreements in KSA. However, this initial movement needs to be incremented into a more structured intervention, so that it can have spillovers and have a systemic impact. For example, in KSA only the National Guards Health system routinely incorporates health economics as a mandatory analysis, while at other centers it is optional. This is an opportunity to grow with exploring the benefits of these techniques.

The role of the pharmaceutical committees was also discussed. Departing from the idea these committees exist to make a rational use of drugs inside the institutions, the Forum elaborated on topics, such as the prioritization of treatments and conditions, the process of work, performing class reviews, and confronting current practice with local/international guidelines.

The pharmacist is a key player in this chain of care. Pharmaceutical care was presented at the Forum as being patient-centered. This means an outcomes-oriented pharmacy practice has the pharmacist to work in concert with the patient and the other healthcare providers to promote health, to prevent disease, and to assess, monitor, initiate, and modify medication use to assure drug therapy regimens are safe and effective. The Forum also highlighted the pharmacist reviews, monitors, and

modifies the therapeutic plan as necessary and appropriate, in concert with the patient and healthcare team.

As far as pharmacoeconomics is concerned, there is also a potential role for the pharmacists. Some areas were enumerated at the Forum, including identifying cost saving interventions, contribution on reduction of adverse reactions through monitoring and proper use, and advising on the reduction of emergency visits to the hospital and readmissions. They can also look on oral chemotherapy programs, process improvements, discuss administration methods, or dose rounding. They routinely promote for further savings (e.g., vial sharing). Pharmacists can also play a role in analysis and document evaluation at economic evaluations, from budget impact analysis to the full range of cost effectiveness studies.

On a related topic, risk-sharing agreements were also reviewed. In fact, these agreements respond to current challenges, such as demographics trends toward a longer life span or the increasing cost of treatments. They also incorporate the best on scientific knowledge, RWD, and the will to transform healthcare by adding more value to the system, outcomes for patients, and a lesser cost than in a going concern pay for service world to make it affordable.

Hospitals and government bodies will certainly lead the momentum in KSA and will adopt pharmacoeconomics and RSA as part of their operating mode. Value-based healthcare should also inspire more change with significant impact for patients and the healthcare system. This all fits the ongoing health sector 2030 transformation program. The ability to continue to successfully develop RSAs in KSA will certainly be an important step forward in terms of patient access to treatments, particularly but not exclusively in the field of oncology, and a gain in the efficiency of healthcare.

CONCLUSIONS

The Forum succeed at gathering a selected group of key opinion leaders with international experience and training, and was able to produce conclusions on the way to move forward to optimize healthcare spending and to promote a rational use of medications, while giving access to drugs for patients that will benefit from them.

The challenges ahead and the way the participants are preparing for them were captured in the brilliant presentations. Some of those challenges included the increase in drug expenditure, the management of competing drugs in similar classes, and the need for critical and technical appraisal of the studies that support actual decisions. From the perspective of healthcare players, they need to invest in better understanding of how to use RWD and to produce/employ local drug utilization data, from databases or the electronic health records, to evaluate post adoption, how to better measure, acknowledge, and incentivize the contribution of the local healthcare professionals to pharmacoeconomics, and possibly more professionals are needed, so this is a booming professional area with strong requirements regarding quality.

As a result, in the near future we will witness the multiplication of use cases in KSA of incorporating formal HTA techniques in decision making, probably an upgrade on methodology and consistency of procedures, stakeholders will learn from each other's experience before setting a body of norms to follow in KSA, and ultimately a combination of HTA with access models. By having providers to work together with other public or private players, and with the suppliers in access agreements, HTA tools will be mandatory on the side of providers and will have a decisive role at shaping the final terms of these contracts. The outcome will be a negotiated solution where the goal must be optimal outcomes at the lowest possible cost. Payers can also have a relevant contribution, by enforcing contract terms that incentivize good practices and linking payment to outcomes by using value frameworks to structure in dialogue and sustainable delivery models.

The potential of pharmacoenomics is huge at improving healthcare in the region. The agenda is to move forward with quality data captured for the studies and decisions. Registries should also be fostered, as part of a process to prefer local RWD to imported/borrowed metrics, and also to appreciate the work of the healthcare professionals of the Saudi system. There is a need for closer and continued collaboration between the different parties, along with a supporting infrastructure for information sharing, the data standards, and the continuous training of all those involved in pharmacoeconomics through hospital and university programs.

In summary, health economics, pharmacoeconomics, and outcomes research are growing fields of work worldwide. They promise to shed some light on decisions, on established methodologies, and to curb the rising trend of costs in healthcare. Furthermore, they are flexible and can be applied to the analysis of single drugs or to other forms of more complex technologies, such as protocols, pathways, or a combination of treatments across time. These topics will certainly have great development in the region. And the first players to master it, will have a competitive advantage and the opportunity to make a greater difference in patients' lives.

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