

World Health Summit, Berlin

“Improving Processes for Access to Medicines – Developing Stronger Collaboration and Dialogue”

Summary of discussions of a workshop held on 10 October 2016

On 10 October 2016 in the framework of the World Health Summit, a distinguished group of panelists came together to discuss the impact of greater and more structured dialogue between pharmaceutical manufacturers and national agencies responsible for the evaluation and funding of pharmaceuticals.

Every healthcare system is faced with the challenge of both incentivizing more innovation while also enabling appropriate access in a sustainable manner. It is imperative that healthcare systems can evaluate what the implications are for their system of innovations well before reaching the marketing authorization stage. Equally, it is imperative for industry to maintain its efforts to be a partner in achieving access for all.

Processes for horizon-scanning and processes for structured dialogue that involve relevant stakeholders, including manufacturers, early on have emerged over the past years as important tools to manage these requirements.

The Priority Medicines report developed by the WHO in 2004 and updated with the support of the European Commission in 2013, sought to look at pipeline scanning from a public health perspective and address the gaps that should be filled through R&D. The work done on Priority Medicines informed the IMI strategic research agenda and thought should be given to renewing this effort going forward. At the same time, one should also not lose sight that R&D is a complex and often unpredictable process. We can all agree that new approaches to address diseases such as Alzheimer’s are absolutely needed, but it is very difficult to project whether and when this will be delivered.

There are also capability gaps in healthcare systems’ preparedness to anticipate and manage the integration of disruptive technologies. Some of these gaps can be addressed by building adequate frameworks that enable systems to identify early what has a potential high benefit for patients. The notion that authorities should be able to give very early guidance to industry on what pipeline projects are interesting for public health is what we should aspire to, but it will also require consistency over time.

It is encouraging to see a growing consensus, expressed by the panelists, on the need for an efficient system of parallel scientific advice at European level involving the national Health Technology Assessment (HTA) bodies, the European Medicines Agency and the manufacturer. Companies are not able to fulfill every national demand on clinical evidence and an early discussion would help clarifying and where possible aligning evidence needs while giving Europe a stronger voice towards manufacturers operating at a global level.

A more systematic approach in parallel scientific advice should as a priority seek to integrate and streamline different needs in terms of evidence that is relevant for healthcare systems beyond those needed for regulatory requirements. Solid governance in the process can help address this in a constructive and viable manner. Post-launch we need further instruments in place that can address evidentiary needs and here the reflection on adaptive pathways should continue.

There are a number of challenges that will need to be tackled in this area. Panelists focused particularly on the difficulty to manage the balance between additional evidence needs against patient access to a promising new drug, between need for data and results expected. Also, not all HTA agencies will look at real-world evidence with the same level of enthusiasm and we are still far from a genuine system of cross-border data collection infrastructure in Europe. During the discussions, a strong appeal was launched to manufacturers to invest in generating post-marketing-evidence, e.g. registers or observational studies, especially when evidence at market access has gaps (e.g. products under an adaptive pathway).

Panelists also outlined some of the key success factors of a future system for parallel scientific advice. Part of an efficient system will be a standing committee of HTA agencies at EU level in which countries with a strong experience of scientific advice must be represented. Participation of the EMA in the dialogue process is essential. And sustainable financing, for example through a fee-for-service approach, plays a key role in ensuring efficiency.

Thinking of the broader context within which this type of early scientific dialogue takes place, panelists hinted at the balance that has to be achieved in the way HTA is used in decision-making. While in the first instance a technical process, it cannot be completely disassociated from the overall political and socio-economic context within which it is used and which will vary from country to country. Decisions taken in healthcare are taken in a context of political accountability. Transparency in the system and good governance are felt to be of particular relevance to manage these inherent tensions.

The Pharmaceutical industry strives towards a system where manufacturers are paid on the basis of outcomes. Paraphrasing a key opinion leader in the field of HTA, one panelist stressed that while we routinely apply evidence-based decision making, what industry needs to strive towards is decision-based evidence generation. Budget-holders need to make decisions with the patient at the center. Well-functioning dialogue mechanisms that efficiently align the views of national evaluation agencies will be a key tool in optimizing evidence generation.

The session was chaired by Dr. Ricardo Baptista Leite, Member of Parliament, Portugal, and Richard Bergström, EFPIA and featured the following panelists:

- Niklas Hedberg, TLV (Sweden)
- Dr. François Meyer, HAS (France)
- Dr. Thomas Müller, GBA (Germany)
- Dr. Pieter Stolk, Utrecht University (The Netherlands)