THE POWER OF PARTNERSHIPS
TO REALISE THE EU PHARMACEUTICAL STRATEGY

By: Karl Cogan and Lorraine Nolan

Summary: The future success of the European Union (EU) Pharmaceutical Strategy is inextricably linked to the establishment of new, or strengthening of existing partnerships between key stakeholders responsible for the delivery of quality, safe and effective medicines. Although one of many concerned players, medicines regulatory agencies will play a critical role as they are positioned at many key intersections along the lifecycle of a medicine that requires engagement with a variety of stakeholders. Greater integration, collaboration and partnership between actors from across the health care and industry sectors is critical to realising the goals and objectives of the EU Pharmaceutical Strategy.

Keywords: Partnerships, Innovation, Policy, Medicines regulators

Introduction

Central to the success of the EU Pharmaceutical Strategy is the continued and timely access to quality, safe and effective medicines for European citizens, while also ensuring the European pharmaceutical industry remains an innovative world leader. These overarching goals are ambitious and take into account an ever increasing globalised approach to pharmaceutical development in addition to novel forms of evidence generation, utilisation and assessment while also ensuring a holistic patient-centred approach. Only through collaborative partnerships between patients and their representatives, consumers and health care professionals, academia and research organisations, industry, regulatory authorities, health technology assessment bodies, and pricing and reimbursement agencies, can these laudable goals ever be realised.

The Pharmaceutical Strategy was developed against the background of a well-established medicines regulatory network with the European Commission, the European Medicines Agency (EMA) and the medicines regulatory authorities in the Member States working together to ensure access and availability of safe and effective medicines. Moreover, this strong regulatory network is combined with a robust and competitive pharmaceutical industry. Despite a relatively strong medicines infrastructure in Europe, ever more complex supply chains, rapidly changing global contexts i.e. COVID-19, innovation pipelines that are discordant with public and health system needs, and
medicines shortage issues were some of the challenges the strategy needed to overcome. Given the range of potential threats to the integrity of the European medicines environment, it was clear an extensive number of players were required to address these issues. The new EU Pharmaceutical Strategy ultimately sets a policy environment that requires a renewed sense of shared purpose among key stakeholders and alignment of goals through collaborative partnerships to address the range of issues presented.

Intelligent, data-driven surveillance networks will also inform regulators

The synergies between the EU Pharmaceutical Strategy and emerging themes outlined in the joint European medicines agencies network strategy to 2025 – as adopted by the EMA and the Heads of Medicines Agencies (HMA) – are clear. The alignment of common goals and shared objectives enshrines the partnership between policy makers and regulators who oversee the system. To accomplish shared strategic goals and objectives, the importance of partnership extends to all stakeholders, and is the foundation upon which success will ultimately be achieved. Continued access and availability to life-saving medicines, in addition to supporting and enabling innovation, are central pillars common to both the EMA/HMA and EU Pharmaceutical strategies, respectively. While access, availability and innovation are incredibly important outcomes, the development of productive partnerships between key stakeholders is fundamental in realising the potential of both strategies to deliver for European citizens and the economy alike.

Although medicine regulatory authorities are only one of the many concerned players, they have a critical role as they are positioned at many key intersections along the lifecycle of a medicine that requires engagement with a variety of stakeholders. From initial research and development to post-authorisation safety monitoring, national competent authorities will play a vital role in helping to achieve the goals and objectives of the EU’s Pharmaceutical Strategy. While not an exhaustive list, the following examples provide an overview of how the cross-cutting nature of the EU Pharmaceutical Strategy impacts regulators in the context of establishing new, or strengthening existing partnerships to deliver for European citizens now and into the future.

Future Access to Medicines and the Role of “Big Data”

We have embarked on a digital revolution, where innovative technologies such as wearable and/or implantable sensors can generate an incredible amount of data that captures many aspects of our biology in granular detail, often in real-time. This type of data, for example, might
be collected to monitor the performance of a new therapeutic under real-world conditions to complement traditional large-scale randomised control trials. These data might also be combined with multi-layer ‘omic technologies’, to generate an incredibly personalised biological fingerprint. It is not unrealistic to think that future marketing authorisation applications for medicines might include these integrated sources of real-world evidence to inform regulatory decision making concerning the potential safety and/or efficacy of new therapeutics. As a result, regulators will be required to adapt their processes to appropriately realise the potential of “big data” when determining the benefit-risk analysis of a medicinal product.

To address these challenges, and capitalise on the opportunities, the HMA and EMA have established a Big Data Task Force to review this area and its implications for competent authorities. A comprehensive report was published in 2019, which subsequently led to the establishment of a multidisciplinary joint HMA and EMA Big Data Steering Group with membership from a number of key groups within the regulatory network as well as patient and health care professional representation.

As part of its mandate, the group is tasked with upskilling the European medicines regulatory network (EMRN), i.e. national competent authorities (NCA) with responsibility for medicines and medical device regulation, in big data. This includes the establishment of a training curriculum and strategy informed by analysis of the available knowledge and resources across the network. In recognition of the need to have access to additional expertise, it is envisaged that this will involve collaboration with external experts (including industry and academia) and also consider targeted recruitment of data scientists, omics specialists, biostatisticians, epidemiologists and experts in advanced analytics and artificial intelligence (AI).

Sharing of information, expertise and resources is a central pillar of the EMRN. No individual agency will be able to tackle the potential challenges, or realise the opportunities, represented by the use of big data as real-world evidence. Promoting ‘cluster’ based approaches across the network represents an efficient approach to resource allocation to fully capitalise on the potential utility of new forms of data and methods of assessment. Only through collaborative partnerships between Member States within the network who have already developed expertise, in addition to external experts, can regulators fully embrace the potential of big data and new forms of evidence to facilitate continued access to medicines.

**Stakeholder Engagement and Availability of Critical Medicines**

The COVID-19 pandemic has impacted on almost every aspect of our daily lives, including how medicines regulators operate. Medicines lifecycle management, good practice (GxP) inspections, and establishing mechanisms to support development and accelerated approval of therapeutics and vaccines to protect global public health are examples of challenges that regulators needed to overcome to sustain the health and well-being of citizens. At a basic level, ensuring a continued supply of Covid and non-Covid related medicines was a challenge, requiring coordinated engagement with multiple stakeholders to protect against potential shortages. See **Box 1** for a national example from Ireland on the establishment of the Medicines Critical Assessment Group (MCAG) to mitigate against shortages.

Intelligence generated from the MCAG was also shared with European counterparts, thus creating an integrated intelligence network. The potential of MCAG-like systems to feed into wider regional networks is obvious, and extends beyond the current pandemic environment. The success of intelligence-led oversight of medicines availability and supply is fundamentally built on collaborative partnerships between all stakeholders responsible for health care delivery. Intelligent, data-driven surveillance networks will also inform regulators on how best to develop new procedures that enable greater capability to react, but more importantly pre-empt, potential shortages. Ultimately, integrated partnerships like the MCAG will facilitate continued supply of safe and effective medicines to protect our citizens.

**Vibrant Ecosystems Supporting Innovation**

The pace at which innovative medicines and health technologies are developing represents a real challenge for regulators. Although advance therapeutic medicinal products (ATMPs), convergence between medicines and medical devices, and digitalisation of health care are all examples of innovative technologies with potential to significantly change patient health, it is imperative novel therapies and technologies are effectively regulated while also facilitating safe and timely access. In responding to this challenge, while continuing to enable innovation, it is critical that regulatory authorities establish partnerships with a wide variety of stakeholders to facilitate appropriate regulation from discovery through to approval.

The network performs early stage horizon scanning
scanning for the purposes of identifying disruptive and novel technologies and products. It is not just about identification, but also about determining the policy requirements and expertise required into the future. Access and regular interaction with regulatory bodies throughout the early lifecycle of an innovative medicine through official procedures can help guide clinical development programmes and help developers navigate regulatory requirements.

It is important to realise that for medicines to be considered truly innovative in the context of clinical benefit, they should demonstrate meaningful impact on patient care and well-being at least equal to, but preferably above, currently available therapies. For example, a pharmaceutical company might develop a novel compound targeting a new biological mechanism of action to treat a particular disease. If this compound, however, has no additional benefit compared to existing treatments, can it truly be considered innovative? It is important to disentangle innovative science from innovative care and subsequent clinical utility. While targeting novel biological mechanisms with new compounds is and of itself an innovative approach, without data supporting meaningful clinical benefit beyond existing treatments, it is unlikely to benefit patients.

Conversely, to ensure timely access to promising innovative medicines that deliver clinically meaningful benefits, medicine regulators should foster collaborative partnerships to enhance evidence generation for all actors across the health system. During scientific advice procedures, for example, medicines regulators should work collaboratively with health technology assessment bodies to pre-plan data requirements that satisfies both regulatory assessment but also generate the clinical evidence needed by downstream stakeholders. Greater understanding and appreciation of the requirements of other actors within a broader health system can facilitate access to innovative medicines through efficient and timely generation of necessary evidence.

---

† Clinical utility is a term used in medicine to describe the relevance and usefulness of an intervention in patient care.

### Conclusion

The establishment and maintenance of productive partnerships between multiple key stakeholders is vital for the EU’s Pharmaceutical Strategy to ensure that European citizens have continued access to safe and effective medicines. Synergies between the pharmaceutical strategy and key themes in the joint EMA and HMA European network strategy enshrines partnership between the policy makers and regulators who discharge the system. Greater integration, collaboration and partnership between actors from across the health care system and industry colleagues is key to success.

### References

