



**PHARMACEUTICAL COMMITTEE**  
**11 July 2019**

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**Subject: Pharmaceutical legislation: Challenges and opportunities  
Scenario based brainstorming discussion<sup>1</sup>**

**Agenda item 2b**

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**1. Introduction**

The European Union has been a frontrunner in ensuring that patients have access to safe, innovative and affordable medicines. The pace of innovation has increased in recent years. New scientific and technological pharmaceutical applications, such as the use of artificial intelligence for product development, offer many new possibilities but also pose challenges, particularly for the scientific and regulatory assessment of new medicinal products (MPs).

However, these issues are only one element in a multi-faceted discussion about the current challenges and opportunities in pharmaceutical policy. Key issues include therapeutic added value, contribution to unmet medical needs, access and availability, post-market surveillance, manufacturing and supply chains. They also include transparency of costs and pricing, reimbursement decision making as well as affordability for patients and sustainability of health systems. The international dimension of many of these issues should not be forgotten.

There are many different fora looking at these issues from different perspectives. This includes the European Medicines Agency and the Heads of Medicines Agencies network with their regulatory science/future network strategy. It is opportune to bring this discussion to the Pharmaceutical Committee in order to exchange views directly between Member States and the Commission. It is important to ensure that the various public authorities in the chain work together and that the market authorisation agencies maintain an institutional dialogue at both EU and national level with the organisations in charge of HTA, pricing and reimbursement decision makers and healthcare payers.

This is the first of a series of discussions in the Pharmaceutical Committee addressing the following question: Does the current framework stand up to the challenges and opportunities ahead, and how can we ensure that our pharmaceutical system remains true to its core

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<sup>1</sup> This document has not been adopted by the European Commission and, therefore, it does not reflect an official position of the European Commission.

principles (public health, internal market, innovation, patient access) while taking advantage of new technologies?

## 2. Current regulatory framework

The current legal framework governing medicinal products for human use in the EU is founded on the single market and based on the fact that the EU should take measures to set high standards of quality and safety for medicinal products to serve **public health**.

The EU Pharmaceutical legislation<sup>2</sup> translates these general goals into a comprehensive legal framework governing aspects related to the whole life-cycle of medicinal products, from the initial idea for a new development of an innovative medicine to the administration of the finished product at bedside. It strives to ensure access to affordable, safe and effective medicines for all citizens.

## 3. Technological developments and other drivers

As important scientific and technological breakthroughs mature in the 21st century, it is likely there will be a paradigm shift in pharmaceutical R&D, production, monitoring, sourcing, as well as the patients' and healthcare professionals' roles in the system. The World Economic Forum has described these changes as part of the 4th Industrial Revolution (industry 4.0). This does not affect only the EU, but has a global dimension.

Developments in individual whole **genome sequencing and other –omics** have brought a unique understanding of disease (including predisposition) and have provided tailor-made treatment options.

**Artificial Intelligence** solutions are increasingly able to use advanced algorithms to analyse large amounts of data and identify potential therapies from databases of molecular structures. This possibility can shorten the medicines-production cycles and help the pharmaceutical industry in developing new products through faster and cheaper clinical trials processes. The same technologies can allow real-time monitoring of **real world data** through e.g. **wearables, nano-sensors, electronic health records**. In an ideal scenario, doctors will be able to translate scientific and technological developments in the delivery of care and become co-creators of health care solutions with empowered patients. Holistic treatment strategies combining the traditional “pill” with **digital health apps**, services and feedback mechanisms may become part of standard practice.

These digital developments can help foster a **new paradigm in clinical trial design** from a pre-clinical testing to a more post-approval framework using online patient communities.

New developments in **3D printing** technologies can allow for more effective medicine absorption and better dosage regulation. Such technologies will also bring new models in the production and distribution of pharmaceuticals. Innovations in the field of **robotics** can offer new solutions in medication dispensing and inventory. **Blockchain technology** can further increase security and fight the circulation of falsified medicines.

This is only a snapshot, but underlines the pace of innovation and provides a glimpse of the developments that may affect the pharmaceutical regulatory system. While all these

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<sup>2</sup> Primarily Directive 2001/83/EC and Regulation (EC) No 726/2004

developments may lead to breakthrough therapies, still there are many unmet needs (vaccines, antibiotics) and promising therapies are not always reaching the patient.

It would be important to position this scenario-based discussion in the broader context set by the interplay between accessibility, availability, affordability and sustainability.

#### **4. Points for discussion/questions**

1. What other policy challenges may lie ahead?
2. How flexible is the current system to accommodate these changes?
3. Are there any limitations in our current regulatory system?

#### **5. The regulator in 2030 - possible ways forward**

To answer these questions, we ask you to imagine yourself as a regulator in the year 2030.

In this hypothetical future pharmaceutical landscape, the technologies mentioned above and others have matured to a point that companies have changed their structures, practices and investment plans to focus on new business models, new enhanced products using novel technologies in an "industry 4.0" format while the solutions they offer make use of empowered patients and big data. As a regulator, you will assess their medicinal products, which they aim to market worldwide, for authorisation in the EU. The products are assessed or have possibly received authorisations from regulators in a few other jurisdictions whose legislation may have been adapted to face these new developments.

You are operating on the basis of the current EU legal framework (i.e. regulatory requirements, obligations and incentives), complemented by measures building on existing flexibility which tries to take into account new developments such as clinical, scientific and technical; manufacturing, supply and accessibility of medicines; new disease and health threats. It makes use of current flexibilities and tools (such as conditional approval, accelerated assessment, PRIME etc.) soft law, best practices and benchmarking developed together with stakeholders, as well as other relevant actions at national level.

Seeing possible challenges and opportunities for the pharmaceutical framework from this perspective. Would there be stress or tension on the regulatory framework? Do you feel comfortable that we can fulfil the objectives of the framework under these conditions or is additional work necessary? Where do you see the need for priority action and additional work? Where are limitations and bottlenecks?