European Commission

# ROADMAP

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| **TITLE OF THE INITIATIVE** | Pharmaceutical Strategy - Timely patient access to affordable medicines |
| **LEAD DG – RESPONSIBLE UNIT** | DG SANTE, Medicines: policy, authorisation and monitoring |
| **LIKELY TYPE OF INITIATIVE** | Communication |
| **INDICATIVE PLANNING** | *Q4 2020* |
| **ADDITIONAL INFORMATION** | *https://ec.europa.eu/health/human-use/strategy_en* |

## A. Context, Problem definition and Subsidiarity Check

### Context

The EU strives to be a frontrunner in ensuring universal health coverage. People across the EU expect to benefit from equal access to safe, state-of-the-art and affordable new and established therapies. Medicines play an important role in this regard as they offer options for diagnosis, treatment and prevention of diseases.

The unprecedented coronavirus pandemic clearly demonstrates the need to modernise the way the EU ensures access to medicines for its population. It shows the scale of the necessary and coordinated public health responses that are required to tackle such kind of pandemics. It demonstrates the need to have a future-proof and crisis-proof system to ensure timely access to safe, quality and affordable medicines under all circumstances.

The pharmaceutical sector is a major contributor to the EU economy as a knowledge-intensive sector with 842,000 direct jobs and an €91 billion trade surplus in 2018. The global pharmaceuticals market is estimated to grow in the next years, offering a growth potential for EU industry. Innovative technologies such as artificial intelligence (AI) as well as access and analysis of data collected from clinical experience (real world data) are changing the way products are developed and have the potential to transform therapeutic approaches and business models.

At the same time, the EU has an ageing population, a rising burden of diseases and is faced with emerging health threats such as the coronavirus. To ensure the financial and fiscal sustainability of Member States' health systems, the new therapies need to be clinically better than existing alternatives as well as cost effective.

Against this backdrop there has been a recurrent debate about access, availability (including shortages) and affordability of medicines.The Council conclusions of 2016 focused the debate on how to reconcile innovation with the need to ensure wide access to innovative products for unmet needs and the financial sustainability of health systems. The European Parliament adopted in 2017 a resolution on possible EU options for improving access to medicines, and in 2018 a resolution on anti-microbial resistance and the need to reduce antibiotics' use.

Therefore, there is a need to build a holistic, patient-centred, forward-looking EU Pharmaceutical Strategy which covers the whole life-cycle of pharmaceutical products from scientific discovery to authorisation and patient access. The strategy should ensure the sound functioning of the internal market and the sustainability of public finances. It will create synergies with relevant EU policies, such as in the areas of research and innovation, industry, competition, environment and chemicals. Coherence will be kept with EU clinical trials and medical devices legislation.

The Commission will launch a Pharmaceutical Strategy for Europe to continue ensuring the quality, safety and efficacy of medicines and reinforcing the sector's global competitiveness. Europe should also make sure that all patients can benefit from innovation while containing the pressure of increasing costs of medicines.

This initiative is in line with the New Industrial Strategy for Europe and the priorities outlined in the European Green Deal, Europe's Beating Cancer Plan, the European Digital Strategy. It takes into account geopolitical considerations.

### Problem the initiative aims to tackle

A number of challenges have been identified:

**1. A rapidly changing global context which can have a major impact on access to medicines in the EU.**

As the coronavirus outbreak has turned into a global pandemic, it has demonstrated the serious exposure of and impact on the EU health and pharmaceutical sectors. It also showcases that European responses to new and emerging health crises do not happen in a vacuum but rather have an important international dimension. Likewise, health crises in one Member State have the potential to spread rapidly across the EU. More structurally, the EU's growing dependency on imports of medicines and active pharmaceutical ingredients due to manufacturing outside the EU may result in shortages in case it is not counterbalanced by a sufficient level of diversification of EU supply chains. This may raise health security risks and is a concern for the EU's strategic autonomy, under normal circumstances but also under a crisis situation.

1) **Unequal access to medicines that are not always affordable for patients and for national health systems across the EU**. Innovative and promising therapies, including cancer medicines, do not always reach the patient due to market failures, high prices issues related to transparency, companies' marketing strategies, and/or pricing mechanisms. Small markets are particularly exposed to these problems. Pricing and reimbursement policies, being a national competence, differ across the EU. Also, many national health systems face long-term fiscal sustainability issues. Lastly, high levels of out-of-pocket payments put pressure on low-income households.
2) **Shortages of medicines**. These often concern off-patent medicines such as antibiotics, cancer medicines and vaccines. Such shortages may compromise patient care and pose a health security risk. The reasons are complex. They range from commercial strategies of pharmaceutical companies, product withdrawals, weak public service obligations, to manufacturing problems, supply chain complexity or specific issues in pricing and reimbursement.
3) **Innovation efforts are not always aligned to public health and health systems' needs**. Innovation does not always correspond to public health and health systems needs. Therapies or medical technologies for major unmet needs such as novel antimicrobials or dementia are not developed because of science limitations or lack of interest from industry to invest. In addition, the lack of a common understanding of the concept of unmet medical needs between stakeholders and decision makers contributes to this problem.
4) **Challenges for the EU pharmaceuticals innovation ecosystem.** Funding on research done in the EU including by smaller, innovative biotech companies is not always translated into commercially exploited innovation for example through EU biotech and investment hubs. Therefore the value generated by European scientific discoveries is often captured elsewhere.
5) **Technological and scientific developments may challenge the regulatory framework and consequently lead to unintended barriers to needs-driven innovation**. Important scientific and technological advances such as gene and personalised therapies, smart health applications, medical technologies, including AI, are transforming the landscape and becoming increasingly integrated as part of overarching therapies. However, the regulatory framework may not be keeping pace with these changes. Existing rules may not be sufficiently equipped, for instance to enable the assessment of medicines combined with self-learning AI, and to clearly define the responsibilities of the business operators for such products. Additionally, the regulatory framework may not be fully adjusted for the use of real world data and complex clinical trials for the authorisation of medicines. This may become a barrier to the timely access of EU patients to state-of-the-art products. In addition, fragmented granting procedures in the Single Market of some pharmaceutical incentives (e.g. supplementary protection certificates) hamper innovation. Health systems may need to be better equipped to ensure deployment and uptake of innovative solutions.
6) **The way environmental risks are addressed needs to be improved**. To respond fully to the objectives of a green economy, the regulatory framework needs to address the environmental implications of production, use and disposal of medicines. One of the major challenges is increasing antimicrobial resistance.

**Basis for EU intervention (legal basis and subsidiarity check)**

Articles 114 and 168 of the Treaty on the Functioning of the European Union (TFEU), give the EU the competence to set measures establishing an internal market and setting high standards of quality and safety of medicinal products, and to support, coordinate or supplement the actions of EU Member States for the protection and improvement of human health ensuring a high level of protection. Providing patients across the EU with medicines of demonstrated safety, quality and efficacy is a core principle of the EU pharmaceutical regulatory framework which covers the lifecycle of medicines.

Availability of and equal access to affordable medicines for citizens across the Union depends on the smooth functioning of the EU internal market but also national decisions after regulatory approval of medicines. Key issues relate to pricing, reimbursement, application of procurement rules and health systems financial and fiscal sustainability. These issues are primarily dealt with at the national level as they relate to national policies and/or competences. At the same time, coordinated actions and enhancing Member States' collaboration efforts may prove beneficial to achieve the best results and may lead to synergies and mutual learning.

## B. What does the initiative aim to achieve and how

The overall goal of the initiative is to **help ensure Europe's supply of safe and affordable medicines to meet patients' needs and support the European pharmaceutical industry to remain an innovator and world leader**. Being linked to the new Industrial Strategy, this initiative aims to boost the global competitiveness of the

EU the pharmaceutical manufacturing value chain and to secure the EU's strategic autonomy in this area. The strategy will build on an evidence-based assessment and review of the existing regulatory framework and policy, aiming towards a system that is future-proof and that consistently addresses all levels of the value chain, from R&D to authorisation and access of patients to medicines. It will take into account scientific and technological advances and the necessity to ensure environmental sustainability.

The strategy will also seek to address market failures (e.g. lack of new antimicrobials) and build on initiatives to support the financial and fiscal sustainability of health systems. It is linked to the Commission's industrial strategy and will create synergies with other policies and key Commission priorities (e.g. the EU Green Deal, Europe's beating cancer plan, research and innovation, competition, intellectual property rights).

The pharmaceutical strategy will examine the need for legislative and non-legislative actions and EU investments. Legislative actions may encompass follow up to the initiatives which are already in preparation, such as the review of the legislation on medicines for rare diseases and children (Orphan and Paediatric Regulations), the legislation on fees for the European Medicines Agency. It could also include a targeted evaluation and subsequent review of the basic pharmaceutical legislation[1], and to the extent necessary, other legislative acts. Legislative initiatives will be complemented by non legislative actions. EU investments would include various programmes, such as Horizon Europe (funding for researchers, to innovative companies and establishing European Partnerships with industry actors), InvestEU, Digital Europe Programme.

The following specific objectives will be pursued through legislative and non-legislative actions:

- **Ensure greater access and availability of pharmaceuticals to patients**;

  Review incentives and obligations for innovation, market launch/entry and continuous supply of products. Promote regulatory and administrative simplification (e.g. through the better use of electronic product information and multilingual packs) and increase the overall transparency in the sector, to support need-driven innovation for patients. Ensure quality, safety and efficacy of products, enhance oversight of global manufacturing and clarify responsibilities to ensure quality of medicines. Contribute to the sound functioning of the internal market including for generics and biosimilars.

- **Ensure affordability of medicines for patients and health systems financial and fiscal sustainability;**

  Support, through non-legislative measures, EU co-operation on issues related to evaluating cost effectiveness and measuring added therapeutic value, to pricing and reimbursement, procurement practices while enhancing Member State co-operation through information sharing and best practices. Strengthening EU cooperation on health technology assessment (HTA) as envisaged in the Commission's legislative proposal.

- **Enable innovation including for unmet medical needs in a way that harnesses the benefits of digital and emerging science and technology and reduces the environmental footprint**;

  Support breakthrough innovation through timely approval and market access and also by way of innovative procurement. Also support, inter-disciplinary cooperation to facilitate need-driven development of new products and services and their acceptance in clinical practice, particularly in areas of unmet needs. Review procedures for accelerated development and assessment of medicines for major public health needs taking into account novel technologies to respond timely to public health threats such as the coronavirus pandemic, without compromising on patients safety. Also in case of emerging health threats, reinforce the mechanisms for cooperation and coordination between the regulatory authorities. Consider opportunities brought by gene therapies and personalised medicine, e.g. for cancer and neurodegenerative diseases. At the same time, reduce use of resources, emissions, degradation and pollution throughout the whole life cycle of pharmaceuticals and promoting the rational use of medicines. Support development of skills and capacity building.

- **Support EU influence and competitiveness on the global level, reduce direct dependence on manufacturing in non-EU countries, seek a level playing field for EU operators**.

  This objective calls for supporting EU regulatory presence and global influence aiming to achieve a level playing field for EU companies through harmonised international standards of quality and safety of medicines and by addressing environmental risks. Encourage and support EU manufacturing capacity for active pharmaceutical ingredients and pharmaceutical starting materials – crucial elements of a single chemical-pharmaceutical strategic value chain. Build on the lessons learnt from the coronavirus pandemic.

## C. Better regulation

### Consultation of citizens and stakeholders

The preparation of the Strategy will be supported by comprehensive consultation activities with a wide variety of stakeholders, including associations representing patients and consumers, healthcare professionals, the

[1] Directive 2001/83/EC and Regulation (EC) 726/2004

pharmaceutical industry, including SMEs, academia. It will include consultations with Citizens, Member State national competent authorities responsible for pharmaceutical policy, HTA bodies, pricing and reimbursement authorities, payers, public buyers, international partners and organisations and the European Parliament. The Roadmap will be actively promoted to stakeholders notably using the EU Health Policy Platform and through representative organisations. The feedback received will allow to better target the focus of the strategy and of its initiatives.

The consultation activities will include:

- **Open public consultation** for 12 weeks will be available on 'Have Your Say' .
- **Member States, the EMA and EEA partners** will be consulted in the Commission's Pharmaceutical Committee on their expectations on the long-term strategy. Member State experts in pricing, reimbursement and public payers authorities will be consulted in other relevant fora as required.
- Regular exchanges with the relevant committee in the **European Parliament** will take place
- **Additional consultations** with relevant stakeholders will be organised for feedback and input on the strategy or on individual pillars of the strategy. This will include outreach to patient, consumer and civil society organisations, healthcare professionals, researchers, environmental organisations, and pharmaceutical businesses and their associations.
- A synopsis report, summarising the results of all consultation activities will be published on the consultation page once all consultation activities are closed.

**Evidence base and data collection** [max 10 lines]

The strategy and its underlying analysis will build on:

- the completed study on the economic impact of supplementary protection certificates, pharmaceutical incentives and rewards in Europe,
- initial results of other studies and ongoing data collection, namely:
    - studies concerning the evaluation of medicines for rare diseases and children, and
    - study on "accessibility of pharmaceutical care and sustainability of pharmaceutical spending: an analysis of sales data" (IQVIA MIDAS sales data),
    - study on marketing authorisation procedures, and
    - evaluation on EMA fees
- analysis by the Commission and by contracted research projects, in addition to the analysis of existing market data.

The Commission will also benefit from the expertise of its Agencies. Finally, national plans and network strategies, like the one of EMA and the heads of national medicines agencies will also form an important contribution to the underlying analysis.

No impact assessment will be carried out for the strategy itself. Actions developed under the strategy with expected significant impacts will become the object of an impact assessment, in line with the better regulation guidelines.

The results of ongoing projects, like the evaluation of the legislation on medicines for children and rare diseases, key principles for the use of electronic product information for EU medicines and action plans (such as action plan for supporting development of medicines for children, European Union strategic approach to pharmaceuticals in the environment, EU action plan on advanced therapy medicinal products (ATMPs)), will inform the strategy and initiatives related to the pharmaceutical legislation.