

Survey on the Pharmaceutical Strategy - Timely patient access to affordable medicines

Fields marked with * are mandatory.

Introduction

The EU strives to be a frontrunner in ensuring universal health coverage. In addition, it is a global leader in healthcare research and development and a major trading partner in pharmaceuticals and medical technologies. 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 therapeutic options for diagnosis, treatment and prevention of diseases.

The unprecedented coronavirus pandemic (COVID-19) clearly demonstrates the need to modernise the way the EU ensures that its citizens get the medicines they need. Although this has been thrown into sharp relief by the coronavirus pandemic, it is not a new problem: even prior to the pandemic we witnessed shortages of essential medicines, such as cancer treatments, vaccines and antimicrobials. This calls for a thorough examination of how the supply chain - from the importing of active ingredients, raw materials, and medicines from third countries to internal EU production and distribution – can be made more secure and reliable.

Securing the supply of medicines is not only about existing therapies. There is also a need to ensure that the European pharmaceutical industry remains an innovator and world leader. Innovative technologies such as artificial intelligence as well as data collected from clinical experience (“real world data”) have the potential to transform therapeutic approaches and the way medicines are developed, produced, authorised and placed on the market and used. Innovation needs to be focused on areas of most need.

At the same time, more must be done to ensure that innovative and promising therapies reach all patients who need them: at present, this is not the case, with patients in smaller markets being particularly affected. Health systems, which are also seeking to ensure their financial and fiscal sustainability, need new therapies that are clinically better than existing alternatives as well as cost effective.

Finally, we are more aware than ever of the need to reduce the environmental footprint of medicines.

All these challenges will be addressed in the forthcoming EU Pharmaceutical Strategy, which should cover the whole life-cycle of pharmaceutical products from scientific discovery to authorisation and patient access.

More information on the context of the initiative, on the challenges identified so far and on the objectives can be found in the roadmap (<https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12421-Pharmaceutical-Strategy-Timely-patient-access-to-affordable-medicines>). Whether you are a concerned citizen or a professional in the area of medicines we would like you to let us know if you share our

objectives, what actions we should focus on and whether there are any additional aspects that we should cover.

After some introductory questions about yourself, the questionnaire continues with questions on the Pharmaceutical strategy.

When replying, please keep in mind that the questions in this survey were developed to address the long-standing issues identified in the EU pharmaceuticals system. These may be related to the problems arising from the coronavirus pandemic but are broader than that. The end of the survey includes dedicated questions on coronavirus related issues.

Please note that in this questionnaire, we do not intend to obtain data relating to identifiable persons. Therefore, in case you will describe a particular experience or situation, please do it in a way that will not allow linking to a particular individual, whether it is you or somebody else.

We thank you in advance for your time and input.

International dependency and manufacturing

The EU is increasingly dependent on active ingredients originating from outside the EU. This has implications, including as regards increasing the risk of quality issues and shortages of medicines. The recent outbreak of COVID-19 shows that a disruption in the pharmaceutical products supply chain originating from outside the EU could present a major health security issue.

1. What type of EU action or initiative do you consider helpful to incentivise the production of active pharmaceutical ingredients for essential medicines (e.g. antibiotics, oncology medicines) in the EU?

800 character(s) maximum

Revision of tendering procedures to include the criterion of supply chain resilience, especially the location of the production sites. One solution could be the creation of a label "medicine made in Europe" which could be used as a requirement in tendering procedures.

The reshoring of APIs production should not be the sole measure taken in this context. The Commission should also explore how to diversify supply sources located externally.

More data must be gathered on supply chain risks to establish exactly where their vulnerabilities lie and how its resilience can be strengthened.

Pharma companies supplying medicines on the EU market should be able to demonstrate their supply chain's resilience and provide contingency plans in case of supply disruptions.

2. What action do you consider most effective in enhancing the high quality of medicines in the EU?

between 1 and 1 choices



Stronger enforcement of the marketing authorisation holder responsibilities

- ☐ Increased official controls in the manufacturing and distribution chain
- ☐ Other (please specify)
- ☐ I don't know

Access to affordable medicines

A shortage of a medicine occurs when there are not enough medicines in a country to treat every patient with a given condition. Shortages can have a big impact on patients if their treatment is delayed because there is no alternative, or the alternative is not suited to their needs.

3. Are you concerned about medicines shortages in the EU?

- ☒ I am concerned
- ☐ I am not concerned
- ☐ I have no particular opinion

If you wish, please elaborate your reply.

500 character(s) maximum

Medicine shortages are a daily experience of doctors, and hospital and community pharmacists.

A medicine shortage means in practice that doctors cannot give the necessary medicines to patients. That can lead to possible delays in patients' treatment, to the need to switch to alternative therapies that could be less effective, to adverse effects and adherence problems or even life threat when a shortage concerns essential medicines.

4. Which actions do you think would have the biggest impact on reducing shortages in the EU?

at most 3 choice(s)

- ☒ Stronger obligations on medicines producers, and other players in the supply chain to ensure medicines are available
- ☒ Transparent information exchange among authorities on medicine stocks available in each country
- ☐ Increased cooperation among public authorities/national governments on shortages
- ☐ Multi-lingual packaging and electronic product information leaflets facilitating purchasing in different countries
- ☐ Providing incentives to companies to increase the production of medicines in the EU
- ☐ Inform on and make available to patients suitable substitutes for medicines that are at risk of shortage

☒ Other (please specify).

Please elaborate your reply.

500 character(s) maximum

Besides the two above points:

Agreeing on common definitions of medicine shortages and essential medicines

Amending tendering procedures (i.a. applying different criteria than price and allowing more than one winner)

Increasing diversification of supply sources and reducing Europe's reliance on external manufacturing
(not only through additional incentives to pharma companies)

Empowering the EMA to monitor and coordinate the response to shortages at EU level

Innovative medicines have to undergo a centralised EU-wide marketing authorisation. Companies often initially market them in a limited number of EU countries. It can take several years before patients in the other EU countries have access to those products.

5. Do you think that companies that apply for and receive an EU-wide marketing authorisation should be required to make that product available in all EU countries?

- ☒ I agree
- ☐ I neither agree or disagree
- ☐ I disagree
- ☐ I don't know

If you wish, please elaborate your reply.

500 character(s) maximum

The EU can contribute to ensuring equal access to medicines in all Member States by subjecting the granting of the marketing authorisation to a commitment on the part of pharmaceutical companies that once authorised, medicinal products will be launched in all EU countries at the same time. Moreover, pharmaceutical companies should not be allowed to subsequently withdraw these products from particular markets for commercial reasons.

In recent years, there has been an [increase](#) in the number of medicines withdrawn from the market upon decisions by the manufacturers.

6. Do you have an opinion on the reasons for these market withdrawals?

- ☒ Yes
- ☐ No

If yes, please elaborate.

500 character(s) maximum

Pharmaceutical companies' decisions to discontinue and withdrawal products from particular (less profitable) Member States' markets are i.a. commercially-motivated and profit-oriented.

7. Are you aware of patients not receiving the medicine they need because of its price?

- ☒ Yes
☐ No

If you wish, please elaborate your reply.

500 character(s) maximum

New medicinal products administered in oncology (e.g., checkpoint inhibitors, CAR-T-cells) marketed at a very high price and therefore not available in many Eastern European countries, hepatitis C treatment Sofosbuvir (Gilead's drug Sovaldi <https://bit.ly/2EI0ngF>) or cystic fibrosis treatment lumacaftor/ivacaftor (Vertex's drug Orkambi <https://bit.ly/2BTjZxr>), among many others.

8. Do you think that medicine prices are justified, taking into consideration the costs associated to their development and manufacturing?

- ☐ Yes
☒ No
☐ I don't know

If you wish, please elaborate your reply.

500 character(s) maximum

Transparency (encompassing not only R&D and manufacturing costs but also incentives) is a prerequisite for pharmaceutical pricing. In this respect, the final price of a drug should be reasonable and connected to the disclosed public investment and the manufacturing costs. Approach to medicine pricing should be multidimensional, based on HTA, where various factors are taken into account from the therapeutic added value to the ability to pay and the cost of drugs.

High prices for new medicines put pressure on public health spending. The costs for research and development are not publicly disclosed and there is no agreement on how to calculate such costs. In certain cases, some EU countries join forces to increase their negotiating power when discussing prices with pharmaceutical companies. Individual pricing decisions in some EU countries may affect others. As an example, some EU countries limit the prices of medicines by linking that price to average prices in other EU countries (we call this "external reference pricing"- ERP). Because of ERP, a pricing decision in one EU country can inadvertently affect the prices in others. Once patents and other forms of market protection expire, generic and [biosimilar](#) medicines can enter the market and compete with the existing ones, this also typically brings down prices. Finally, there are plans to strengthen support to EU countries

to work with each other on the clinical effectiveness of new medicines compared to existing alternatives, simply put how much better a medicine works compared to another one. This is part of the so called "health technology assessment" process.

9. What are the most effective ways the EU can help improve affordability of medicines for health systems?

at most 3 choice(s)

- ☒ Support the EU countries in better assessing and/or evaluating the value of medicines, meaning the effectiveness of a (new) medicine compared with existing ones
- ☒ Help EU countries share experiences and pool expertise on pricing and procurement methods
- ☐ Better coordination among EU countries to ensure that pricing decisions taken by one EU country do not lead to negative impacts on patient access in another EU country
- ☐ Facilitate, market entry and a healthy market functioning for generics and biosimilars
- ☐ More transparency on how the cost of a medicine relates to the cost of its research and development
- ☒ There should be a fair return on public investment when public funds were used to support the research and development of medicines
- ☐ I don't know
- ☐ Other

Innovation in early development and authorisation

The European Commission actively supports health research and development through various funding mechanisms (e.g. Multiannual Financial Framework, [Horizon 2020](#), [Innovative Medicines Initiative](#) partnership) and through collaborations between academia, healthcare systems and industry. Furthermore, the EU pharmaceutical legislation includes incentives to stimulate the development of innovative new medicines in areas such as paediatric and rare diseases; and market exclusivity rights to industry.

10. What actions at EU level do you consider most effective in supporting innovative research and development of medicines?

at most 3 choice(s)

- ☒ Make the legislative framework more adaptive to new technologies and advances in science
- ☒ Provide more public funding for research

- ☐ Support (including through funding) private-public partnerships
- ☐ Support (including through funding) the creation of start-ups in medical research
- ☐ Foster research collaboration between universities, research centres and industry
- ☐ Provide research and development incentives in the form of intellectual property or market exclusivity rights for pharmaceutical companies investing in research
- ☐ Simplify the requirements for the conduct of clinical trials
- ☒ Other (please specify)
- ☐ I don't know

Please elaborate your reply.

100 character(s) maximum

Transparent and balanced governance of the EU's innovation's initiatives.

Expected return on investment in research and development for the pharmaceutical industry depends also on the expected volume of sales; this seems to be one of the root causes of limited availability of certain medicines (e.g. medicines for rare diseases or medicines for children).

11. What do you consider are the most effective actions related to research and development of medicines in areas where there are limited or no therapeutic options (unmet needs)?

at most 3 choice(s)

- ☐ Provide market protection (protect a new medicine from competition)
- ☐ Provide intellectual property protection
- ☐ Provide data protection (protection of the data related to a medicine's clinical trials)
- ☒ Agree on a common understanding on what are the areas of unmet need in the EU
- ☒ Funding more targeted research at EU level
- ☐ Funding more targeted research at national level
- ☐ Provide national schemes to support companies economically
- ☐ I don't know / no opinion



Other (please specify)

Please elaborate your reply.

100 character(s) maximum

Agree on a common definition of “innovative medicine”

Attach conditionalities to public funding

The health sector is becoming more digitised, thanks to the increased availability and collection of health data from sources such as electronic health records, patient and disease registries and mobile apps (i.e. real world data) and through the use of artificial intelligence (AI) (i.e. systems that display intelligent behaviour and the use of complex algorithms and software in the analysis of complex health data). These developments, combined with real world data are transforming health, including the discovery of medicines.

12. Which **opportunities** do you see in digital technologies (such as artificial intelligence and use of real world data) for the development and use of medicines?

600 character(s) maximum

Getting new insights (e.g. potential new indications or a different safety profile in certain patient subgroups) from data assembled from various sources (real-world data or completed and ongoing studies) and analyzed applying AI-powered technology.

Increasing the statistical power through more frequent data collection. Reducing the costs of the development of innovative medicines.

Optimizing clinical trials operations and improving patient care throughout them.

Improving manufacturing process and providing advanced analysis of production sites.

13. Which **risks** do you see in digital technologies (such as artificial intelligence and use of real world data) for the development and use of medicines?

600 character(s) maximum

Real-world data should be only considered as complementary to randomized clinical trials and in no case, it should be promoted as their replacement.

It is of utmost importance to have in place appropriate framework ensuring the quality, robustness, reliability and usefulness of collected sensitive health data and also guarantee the confidentiality and privacy of patient data.

Performance transparency and assessment of AI-powered technology is needed to ensure its efficiency, trustworthy and adherence to legal and ethical principles and values.

Continuous manufacturing, advanced process analytics and control, 3D printing and portable/modular systems, may revolutionise the way medicines are manufactured.

14. Are you aware of any obstacles in the EU in taking advantage of technological progress in the manufacturing of medicines?

- ☐ Yes
- ☐ No
- ☒ I don't know

Clinical trials are investigations in humans to discover if a new medicine is safe and effective. Clinical trials can also be used to test if a new treatment is more effective and/or safer than the standard treatment.

Finally, so called “pragmatic clinical trials” can be conducted to compare the safety and effectiveness of different standard treatments in real world setting.

15. How could clinical trials in the EU be driven more by patients’ needs while keeping them robust, relevant and safe for participants?

at most 3 choice(s)

- ☐ By providing more national support for the conduct of so-called “pragmatic trials” with the aim to optimise treatment to patients
- ☐ By better coordination for larger trials comparing different treatment strategies (covering medicines and other treatments such as surgery, radiotherapy, physiotherapy)
- ☒ By providing support for non-commercial organisations to conduct clinical trials in fields where financial interest is weaker
- ☒ By involving patients’ experiences in early phases of medicine design (e.g. factor-in how the disease affects their lives and develop medicines to target symptoms that are particularly important to patients)
- ☐ By designing more trials that collect information on medicine tolerability or the impact of a treatment on the quality of life
- ☐ By taking into consideration during the design of a trial the burden of trial participation on patients’ life
- ☒ Other (please specify).

100 character(s) maximum

By including children, elderly people, and women in appropriate proportion in clinical trials

Certain medicines are developed based on genes, cells or tissue engineering. Some of these products are developed in hospitals. These are covered by the notion of advanced therapy medicines.

16. Is the current legal framework suitable to support the development of cell-based advanced therapy medicines in hospitals?

- ☐ I strongly agree
- ☐ I partially agree
- ☒ I disagree
- ☐ I don't know

* If you responded partially agree or disagree, please provide examples of changes that, in your view, would be required to support the development of these products.

500 character(s) maximum

Special emphasis should be put on programs assuring cell-based ATMPs' quality, efficacy and safety (demonstrated by clinical data) when they are administered.

Environmental sustainability of medicines and health challenges

Residues of several medicines have been found in surface and ground waters, soils and animal tissues across the Union. As of yet, no clear link has been established between medicine residues present in the environment and direct impacts on human health. However, the issue cannot be ignored and there is a need for a precautionary approach.

17. What actions at EU level do you consider most effective in limiting the negative environmental impact of medicines?

at most 3 choice(s)

- ☒ Cleaner manufacturing processes
- ☐ Enhanced application of the polluter pays principle
- ☐ Review the way the Environment Risk Assessment of a medicine is conducted and its consequences on the authorisation process
- ☐ Clear labelling of environmental risks to allow informed choices among equivalent therapeutic options
- ☐ Reference to environmental risks in advertising for over-the-counter medicines
- ☐ Make medicines known to pose an environmental risk available by prescription only

- ☒ Strict disposal rules for unused medicines
- ☐ Prescribe medicines only when it is absolutely necessary (more prudent use)
- ☒ Medicines dispensed to patients in the quantity actually needed (e.g. number of pills, volume of solution)
- ☐ Enhanced wastewater treatment if certain residues could be better removed
- ☐ Other (please specify)

Antimicrobial resistance (AMR) is the ability of microorganisms (such as bacteria, viruses, fungi or parasites) to survive and grow in the presence of medicines. It reduces progressively the effectiveness of antimicrobials and is caused, among other things, by extensive and improper use of antimicrobial medicines. Antimicrobials include antibiotics, which are substances that fight bacterial infections. AMR can lead to problems such as difficulties to control infections, prolonged hospital stays, increased economic and social costs, and higher risk of disease spreading. AMR is one of the most serious and urgent public health concerns.

18. Which actions do you think would have the biggest impact on fighting AMR concerning the use of medicines for patients?

at most 3 choice(s)

- ☒ More prudent use of antimicrobials (if necessary through restrictions on prescriptions)
- ☐ Improve the treatment of wastewater and/or manure to lower the levels of antimicrobials
- ☒ Raise citizens' and healthcare practitioners' awareness by informing them on appropriate use of antimicrobials and the correct disposal of unused medicines
- ☐ Introduce an obligation to use diagnostic tests before prescribing antimicrobials, for example to verify whether it is a bacterial infection before prescribing antibiotics and to define the most adequate antibiotic
- ☐ Public finance research and innovation on new antimicrobials, their alternatives and diagnostics
- ☐ Encourage public health campaigns that prevent infection through better general health including increased immunity
- ☐ Encourage public health campaigns that prevent infection through the use of vaccines
- ☐ Encourage better hygiene measures in hospitals
- ☒ Other (please specify)
- ☐ I don't know

Please elaborate your reply.

100 character(s) maximum

Address the market failures in antibiotic R&D
Promote One Health dialogue and AMR-related funding

Innovation in antimicrobials is limited. For example, no new classes of antibiotics have been discovered for decades. Restricting the use of antibiotics to minimise the risk of developing resistance is a commercial disincentive for investment, as potential investors are concerned that their investment will not be profitable.

19. Where, in your view, should the EU focus its support for the creation of new antimicrobials or their alternatives?

at most 2 choice(s)

- ☒ Support academia for researching/discovering new antimicrobials or their alternatives
- ☐ Support industry for developing new antimicrobials or their alternatives
- ☒ Provide specific support to small and medium-sized enterprises (SMEs)
- ☐ Other (please specify)
- ☐ I don't know

Health threats such as the coronavirus disease test the limits of public health systems, the pharmaceutical industry and of the pharmaceutical legislation. From the beginning of the coronavirus (COVID-19) pandemic, the EU has taken measures to coordinate a [response](#), which includes actions ensuring the availability of medicines.

20. How has the coronavirus (COVID-19) pandemic affected you in relation to access to medicines and treatments?

600 character(s) maximum

The pandemic has aggravated medicine shortages in the EU by causing additional disruptions of the supply chain and by resulting in increased demand for some medicines in clinical trials for COVID-19 and off-label use. The national updates from CPME members gathered over the past months indicated shortage or risk of shortage of sedatives, painkillers, muscle relaxants, antibiotics, antivirals and antimalarials needed to treat COVID-19 patients. The pandemic has brought home the EU's long-existing structural problems and just how fragile and intransparent the supply chains of medicines are.

21. In your opinion and based on your experience, what can the EU do to prepare for and manage such a situation better in the future in relation to pharmaceuticals?

Empower EMA to monitor and coordinate medicines' availability and supply at EU level.

Ensure equitable supplies across the EU (manage stockpiling of essential/strategic medicines and other supplies (medical devices, PPE) at EU level and increase supplies from "just-in-time" to "just-in-case").

Expand the possibility of joint procurement of medicines and other supplies.

Strengthen the resilience of supply chains, increase diversification of supply sources and reduce Europe's reliance on third country manufacturing.

Strengthen and enforce supply and reporting obligations.

Summary question

22. While the Commission is working on improving the EU pharmaceuticals framework, which areas of work do you find most urgent?

at most 3 choice(s)

- ☐ Improve patients' access to medicines
- ☒ Reduce shortages
- ☒ Help national authorities ensure affordability for patients and increase health systems sustainability
- ☐ Support innovation for unmet needs
- ☐ Use of digitalisation to develop medicines
- ☒ Help reduce anti-microbial resistance
- ☐ Reduce the dependency on essential active ingredients and medicines produced outside the EU
- ☐ Environmental sustainability of medicines I
- ☐ don't know
- ☐ Other (please specify)

23. If you were asked before the coronavirus (COVID-19) pandemic, would you have responded differently to any of the previous questions?

- ☐ Yes
- ☒ No
- ☐ I don't know

24. Is there anything else you would like to add that has not been covered in this consultation?

The strategy must regain the trust in the pharma sector and rebuild the confidence in the patient-centred system that serves public interests. Besides the above areas, it should also address:

The abuse of the current model of pharmaceutical incentives and the industry's manipulation of the patent system by using techniques like "evergreening" to extend exclusivity for drugs and prevent generic competition. The revision of the Orphan Drug Regulation and applying stricter rules for the orphan drug designation. The intransparent R&D model that results in a diminishing rate of real pharmaceutical innovation and high prices.

The indicated by the roadmap revision of the procedures for accelerated medicines' development, new means of their assessments, and fast approval and market access must be undertaken cautiously to adequately take patient benefit and safety aspects into consideration.

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