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Open Public Consultation on the revision of the general pharmaceutical legislation

Fields marked with * are mandatory.

Introduction

On 25 November 2020, the Commission published a Communication on a Pharmaceutical Strategy for Europe.

The Pharmaceutical Strategy identifies flagship initiatives and other actions to ensure the delivery of tangible results. As part of the implementation of the strategy, the Commission is evaluating the general pharmaceutical legislation[1] and assessing the impacts of possible changes in the legislation as described in the relevant inception impact assessment.

This public consultation aims to collect views of stakeholders and the general public in order to support the evaluation of the existing general pharmaceutical legislation and the impact assessment of its revision. It builds further on the public consultation[2] conducted for the preparation of the pharmaceutical strategy for Europe. The replies to that consultation will be taken into account for the revision of the general pharmaceutical legislation. The present questionnaire should be seen as a continuation of that process.

In parallel, the legislation for medicines for rare diseases and children is being revised as well. Separate consultation activities have been carried out for that revision.

This questionnaire is available in all EU languages and you can reply in any EU language. You can pause any time and continue later. You can download your contribution once you have submitted your answers.

A summary on the outcome of the public consultation will be published by the Commission services on the 'Have your say' portal.

We thank you for your participation.

*[1] **Directive 2001/83/EC** of the European Parliament and of the Council of 6 November 2001 on the Community code relating to medicinal products for human use (OJ L 311, 28.11.2001, p. 67)*
***Regulation (EC) No 726/2004** of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency (OJ L 136, 30.4.2004, p. 1)*
[2] A report analysing the results of the pharmaceutical strategy consultation was published in November 2020.

About you

* Language of my contribution

- ○ Bulgarian
- ○ Croatian
- ○ Czech
- ○ Danish
- ○ Dutch
- ◉ English
- ○ Estonian
- ○ Finnish
- ○ French
- ○ German
- ○ Greek
- ○ Hungarian
- ○ Irish
- ○ Italian
- ○ Latvian
- ○ Lithuanian
- ○ Maltese
- ○ Polish
- ○ Portuguese
- ○ Romanian
- ○ Slovak
- ○ Slovenian
- ○ Spanish
- ○ Swedish

* I am giving my contribution as

- ○ Academic/research institution
- ◉ Business association
- ○ Company/business organisation
- ○ Consumer organisation
- ○ EU citizen
- ○ Environmental organisation
- ○ Non-EU citizen

- Non-governmental organisation (NGO)
- Public authority
- Trade union
- Other

* Which stakeholder group do you represent?

- Individual member of the public
- Patient or consumer organisation
- Healthcare professional
- Healthcare provider organisation (incl. hospitals, pharmacies)
- Healthcare payer
- Centralised health goods procurement body
- Health technology assessment body
- Academic researcher
- Research funder
- Learned society
- European research infrastructure
- Other scientific organisation
- Environmental organisation
- (•) Pharmaceuticals industry
- Chemicals industry
- Pharmaceuticals traders/wholesalers
- Medical devices industry
- Public authority (e.g. national ministries of health, medicines agencies, pricing and reimbursement authorities)
- EU regulatory partner / EU institution
- Non-EU regulator / non-EU body
- Other (Please specify)

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* Organisation name

255 character(s) maximum

EFPIA (European Federation of Pharmaceutical Industries and Associations), https://www.efpia.eu/

* Organisation size

- ○ Micro (1 to 9 employees)
- ◉ Small (10 to 49 employees)
- ○ Medium (50 to 249 employees)
- ○ Large (250 or more)

Transparency register number

255 character(s) maximum

Check if your organisation is on the transparency register. It's a voluntary database for organisations seeking to influence EU decision-making.

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* Country of origin

Please add your country of origin, or that of your organisation.

- ○ Afghanistan
- ○ Åland Islands
- ○ Albania
- ○ Algeria
- ○ American Samoa
- ○ Andorra
- ○ Angola
- ○ Anguilla
- ○ Antarctica
- ○ Antigua and Barbuda
- ○ Djibouti
- ○ Dominica
- ○ Dominican Republic
- ○ Ecuador
- ○ Egypt
- ○ El Salvador
- ○ Equatorial Guinea
- ○ Eritrea
- ○ Estonia
- ○ Eswatini
- ○ Libya
- ○ Liechtenstein
- ○ Lithuania
- ○ Luxembourg
- ○ Macau
- ○ Madagascar
- ○ Malawi
- ○ Malaysia
- ○ Maldives
- ○ Mali
- ○ Saint Martin
- ○ Saint Pierre and Miquelon
- ○ Saint Vincent and the Grenadines
- ○ Samoa
- ○ San Marino
- ○ São Tomé and Príncipe
- ○ Saudi Arabia
- ○ Senegal
- ○ Serbia
- ○ Seychelles

- Argentina
- Armenia
- Aruba
- Australia
- Austria
- Azerbaijan
- Bahamas
- Bahrain
- Bangladesh
- Barbados
- Belarus
- (•) Belgium
- Belize
- Benin
- Bermuda
- Bhutan
- Bolivia
- Bonaire Saint Eustatius and Saba
- Bosnia and Herzegovina
- Botswana
- Bouvet Island
- Brazil
- British Indian Ocean Territory
- British Virgin Islands
- Brunei
- Ethiopia
- Falkland Islands
- Faroe Islands
- Fiji
- Finland
- France
- French Guiana
- French Polynesia
- French Southern and Antarctic Lands
- Gabon
- Georgia
- Germany
- Ghana
- Gibraltar
- Greece
- Greenland
- Grenada
- Guadeloupe
- Guam
- Guatemala
- Guernsey
- Guinea
- Guinea-Bissau
- Guyana
- Haiti
- Malta
- Marshall Islands
- Martinique
- Mauritania
- Mauritius
- Mayotte
- Mexico
- Micronesia
- Moldova
- Monaco
- Mongolia
- Montenegro
- Montserrat
- Morocco
- Mozambique
- Myanmar/Burma
- Namibia
- Nauru
- Nepal
- Netherlands
- New Caledonia
- New Zealand
- Nicaragua
- Niger
- Nigeria
- Sierra Leone
- Singapore
- Sint Maarten
- Slovakia
- Slovenia
- Solomon Islands
- Somalia
- South Africa
- South Georgia and the South Sandwich Islands
- South Korea
- South Sudan
- Spain
- Sri Lanka
- Sudan
- Suriname
- Svalbard and Jan Mayen
- Sweden
- Switzerland
- Syria
- Taiwan
- Tajikistan
- Tanzania
- Thailand
- The Gambia
- Timor-Leste

- Bulgaria
- Burkina Faso
- Burundi
- Cambodia
- Cameroon
- Canada
- Cape Verde
- Cayman Islands
- Central African Republic
- Chad
- Chile
- China
- Christmas Island
- Clipperton
- Cocos (Keeling) Islands
- Colombia
- Comoros
- Congo
- Cook Islands
- Costa Rica
- Côte d'Ivoire
- Croatia
- Cuba
- Curaçao
- Cyprus
- Heard Island and McDonald Islands
- Honduras
- Hong Kong
- Hungary
- Iceland
- India
- Indonesia
- Iran
- Iraq
- Ireland
- Isle of Man
- Israel
- Italy
- Jamaica
- Japan
- Jersey
- Jordan
- Kazakhstan
- Kenya
- Kiribati
- Kosovo
- Kuwait
- Kyrgyzstan
- Laos
- Latvia
- Niue
- Norfolk Island
- Northern Mariana Islands
- North Korea
- North Macedonia
- Norway
- Oman
- Pakistan
- Palau
- Palestine
- Panama
- Papua New Guinea
- Paraguay
- Peru
- Philippines
- Pitcairn Islands
- Poland
- Portugal
- Puerto Rico
- Qatar
- Réunion
- Romania
- Russia
- Rwanda
- Saint Barthélemy
- Togo
- Tokelau
- Tonga
- Trinidad and Tobago
- Tunisia
- Turkey
- Turkmenistan
- Turks and Caicos Islands
- Tuvalu
- Uganda
- Ukraine
- United Arab Emirates
- United Kingdom
- United States
- United States Minor Outlying Islands
- Uruguay
- US Virgin Islands
- Uzbekistan
- Vanuatu
- Vatican City
- Venezuela
- Vietnam
- Wallis and Futuna
- Western Sahara
- Yemen

- Czechia
- Democratic Republic of the Congo
- Denmark
- Lebanon
- Lesotho
- Liberia
- Saint Helena Ascension and Tristan da Cunha
- Saint Kitts and Nevis
- Saint Lucia
- Zambia
- Zimbabwe

The Commission will publish all contributions to this public consultation. You can choose whether you would prefer to have your details published or to remain anonymous when your contribution is published. **Fo r the purpose of transparency, the type of respondent (for example, 'business association, 'consumer association', 'EU citizen') country of origin, organisation name and size, and its transparency register number, are always published. Your e-mail address will never be published.** Opt in to select the privacy option that best suits you. Privacy options default based on the type of respondent selected

* **Contribution publication privacy settings**

The Commission will publish the responses to this public consultation. You can choose whether you would like your details to be made public or to remain anonymous.

- **Anonymous**
  Only organisation details are published: The type of respondent that you responded to this consultation as, the name of the organisation on whose behalf you reply as well as its transparency number, its size, its country of origin and your contribution will be published as received. Your name will not be published. Please do not include any personal data in the contribution itself if you want to remain anonymous.
- **Public**
  Organisation details and respondent details are published: The type of respondent that you responded to this consultation as, the name of the organisation on whose behalf you reply as well as its transparency number, its size, its country of origin and your contribution will be published. Your name will also be published.

☑ I agree with the personal data protection provisions

Looking back

As mentioned in the Inception Impact assessment, the revision aims to tackle the following problems:

- Unmet medical needs and market failures for medicines other than medicines for rare diseases and children;
- Unequal access to available and affordable medicines for patients across the EU;
- The current legislative framework may not be fully equipped to respond quickly to innovation;
- Inefficiency and administrative burden of regulatory procedures;
- Vulnerability of supply of medicines, shortages of medicines;
- Environmental challenges and sustainability;
- Any other issues, which might emerge from the evaluation.

Q1 In your opinion, are there any other issues that should be addressed in this revision?

800 character(s) maximum

The innovative pharmaceutical industry is contributing to the development of over 8,000 new medicines, translating cutting-edge science into transformative treatments.
Today, though, only 22% of global new treatments are originating in Europe. This represents a reversal of the situation just 25 years ago. In parallel, Europe's share of global R&D investment is falling. If research continues to leave Europe, so will the opportunity to deliver the best care to patients. Through a future-proof regulatory framework, Europe will not fall behind in the global race to attract science for the years to come. Reversing the 25-year trend of investments being relocated away from Europe also means that market conditions, like IPR, are predictable and do not change late in development.

Q2 How has the legislation performed in terms of the following elements?

	Very well	Well	Moderately	Poorly	Very poorly	Don't know
1. Fulfilling its public health protection mission for patients and society.	◉	○	○	○	○	○
2. Promoting the development of new medicines, especially for unmet medical needs.	○	◉	○	○	○	○
3. Enabling timely development of medicines at all times, including during crises.	○	◉	○	○	○	○
4. Enabling timely authorisation, including scientific evaluation, of medicines in normal times.	○	○	◉	○	○	○
5. Enabling timely authorisation, including scientific evaluation during crises.	○	◉	○	○	○	○
6. Adapting efficiently and effectively to technological and scientific advancements and innovation.	○	○	◉	○	○	○
7. Ensuring medicines are of high quality, safe and effective.	◉	○	○	○	○	○

8. Addressing the competitive functioning of the market to support affordability.	○	◉	○	○	○	○
9. Ensuring the availability of generic[3] and biosimilar[4] medicines. *[3] "Generic" is a copy of a medicine based on simple or chemical molecules.* *[4] "Biosimilar" is a copy of a medicine based on biological molecules.*	○	◉	○	○	○	○
10. Ensuring that new medicines are timely available to patients in all EU countries.	○	◉	○	○	○	○
11. Ensuring that medicines stay on the market at all times and that there are no shortages.	○	◉	○	○	○	○
12. Ensuring that authorised medicines are manufactured, used and disposed of in an environmentally friendly manner.	○	◉	○	○	○	○
13. Ensuring that the EU system for development, authorisation and monitoring of medicines, including its rules and procedures, is understandable and easy to navigate.	○	○	◉	○	○	○
14. Attracting global investment for medicine innovation in the EU.	○	◉	○	○	○	○

Is there any other aspect you would like to mention, including positive or unintended effects of the legislation, or would you like to justify your replies?

800 character(s) maximum

While we welcome the evaluation of the general EU pharmaceutical legislation as an opportunity to implement learnings from COVID-19 and ensure a competitive regulatory system in Europe (see Q6 for more granularity), access and affordability of medicines are national competences that cannot be solved by EU legislation. To attract investments in Europe, one of the objectives of the Pharmaceutical Strategy, the incentive and regulatory ecosystems need to be predictable and fostering innovation. Our industry has been able to invest in research and development and to deliver new medicines to patients, healthcare systems and society thanks to the stability of the current European incentives' framework. Any attempt to reduce these incentives will accelerate the erosion of Europe's research base.

Looking forward

This section reflects on possible solutions to address the problems identified in the inception impact assessment mentioned in the previous section.

Your contribution will help us in defining the way forward.

UNMET MEDICAL NEEDS

One of the aims of the strategy is to stimulate innovation and breakthrough therapies, especially in areas of 'unmet medical need'.

Regulators, health technology assessment experts and representatives of bodies responsible for reimbursing or paying for medicines ('payers') are discussing a definition or a set of principles for 'unmet medical needs'[5] in order to achieve the objectives of the general pharmaceutical legislation. The discussions reveal different perceptions of what is an 'unmet medical need'. Convergence on this key concept should facilitate the design of clinical trials, generation of evidence and its assessment, and the quick availability on the market of these products and ensuring that innovation matches the needs of patients and of the national health systems.

The purpose of this question is to identify elements that are important in defining what is unmet medical need and in which areas of unmet medical need innovation should be stimulated.

[5] Please note that a similar discussion is taking place in the context of medicines for rare diseases and for children. The concept of 'unmet needs' in the context of rare diseases and children might be slightly differentiated compared to 'unmet needs' in the context of the general pharmaceutical legislation.

Q3 How important are the following elements for defining 'unmet medical needs'?

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Seriousness of a disease.	◉	○	○	○	○	○
2. Absence of satisfactory treatment authorised in the EU.	◉	○	○	○	○	○
3. A new medicine has major therapeutic advantage over existing treatment(s).	◉	○	○	○	○	○
4. Lack of access for patients across the EU to an authorised treatment.	○	○	○	○	◉	○
5. Other (please specify).	◉	○	○	○	○	○

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined elements, or would you like to justify your replies?

800 character(s) maximum

Meeting one of the first three criteria should be sufficient, requiring all to be jointly met will potentially stifle innovation in an area with large consequences for patients and health systems. An inclusive dialogue with patient representatives and industry will allow the constant refinement of a holistic framework recognising the many ways in which UMN manifest. Patient perspectives should be considered in the notion of major therapeutic advantage, defined as a clinically relevant advantage or a major contribution to patient care, e.g. through improved efficacy, better safety profile, ease of self-administration, improved adherence. We caution against using the fourth criteria, as access is a national competence relying on market dynamics, mostly independent from industry's will.

INCENTIVES FOR INNOVATION

The general pharmaceutical legislation guarantees the pharmaceutical innovator, typically a company, regulatory data and market protection for its new medicinal product. This data protection makes sure that another pharmaceutical company cannot re-use the proprietary data of the innovator for 8 years. Market protection makes sure that a generic or biosimilar medicine cannot be marketed until 10 years after authorisation. This dual protection shields a pharmaceutical innovator from generics or biosimilars on the market for 10 years. This protection is part of the EU system of incentives for innovation. The EU regime of i ntellectual property protection provides an additional protection coverage but is beyond the scope of this questionnaire and the revision of the general pharmaceutical legislation.

Q4 What do you think of the following measures to support innovation, including for ‘unmet medical needs’?

	Very important	Important	Fairly important	Slightly important	Not important	Don’t know
1. The current data and market protection periods for innovative medicines: 10 years of market protection, and 8 years of data protection.	◉	○	○	○	○	○
2. Provide different data and market protection periods depending on the purpose of the medicine (i.e. longer period of protection in areas of unmet medical need).	○	○	○	○	◉	○
3. Reduce the data and market protection periods to allow earlier access for generic and biosimilar medicines to the market.	○	○	○	○	◉	○
4. Introduce new types of incentives[6] on top of the existing data and market protection for medicines addressing an ‘unmet medical need’. *[6] Examples of new incentives are a transferable exclusivity voucher or a priority review voucher. A transferable exclusivity voucher would give the legal right to extend the protection time period of any other patented medicinal product, in exchange for the successful regulatory approval of a specified medicine for unmet medical need (e.g. an antibiotic). The voucher would be transferable or saleable, and may impact the turnover and profitability levels of other products in a developer’s portfolio. A priority review voucher gives priority to the assessment of the application of the medicine in question or another medicine in the applicant’s portfolio.*	◉	○	○	○	○	○
5. Early scientific support and faster review/authorisation of a new promising medicine for an unmet medical need.	○	◉	○	○	○	○
6. Public listing of priority therapeutic areas of high unmet medical need to support product development by providing incentives.	○	○	○	◉	○	○
7. Require transparent reporting from companies about their research and development costs and public funding as a condition to obtain certain incentives.	○	○	○	○	◉	○
8. Other (please specify)	◉	○	○	○	○	○

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

A robust and predictable IP and incentives ecosystem will allow the pharmaceutical industry to develop solutions to today UMN, and ensure that Europe continues to be an attractive location for R&D investment and industrial development. Different types of incentives are required, they work side by side and protect various aspects of innovations.
RDP needs to remain strong, predictable, comprehensive and of sufficient duration. Limiting incentives to narrowly defined categories disregards the reality of science. Besides the difficulty in estimating R&D costs accurately, making incentives conditional upon their transparency could lead to inefficiencies and hamper innovation. The value of a medicine should be considered instead. Novel incentives should be studied in case of no viable market.

ANTIMICROBIAL RESISTANCE[7]

Antimicrobial resistance (AMR) is the ability of microorganisms (such as bacteria, viruses, fungi or parasites) to survive and grow over time and no longer respond to medicines making infections harder to treat and increasing the risk of infections, severe illness and death. Antimicrobials include antibiotics, which are substances that fight bacterial infections. Overprescribing, overuse and inappropriate use of antibiotics are key drivers of AMR, leading to harmful health outcomes. The question below is intended to collect opinions on both the incentives for the development of new antimicrobials as well as possible option on their prudent use.

[7] amr_2017_action-plan.pdf (europa.eu).

Q5 Should there be specific regulatory incentives for the development of new antimicrobials while taking into account the need for more prudent use and if so what should they be?

1000 character(s) maximum

Tackling AMR requires a comprehensive set of policies. To drive sustained R&D investments in this field, a new incentive, a transferable exclusivity extension (TEE), is needed. TEE:
Can be implemented via EU legislation
Does not require upfront government funding and is not dependent on countries' economies or political changes
Would address the failure of the current framework by offering an incentive at the scale required to drive greater R&D in new antimicrobials
Would support pharmaceutical companies of all sizes, including SMEs, and would increase the antimicrobial field attractiveness for private financing mechanisms
Respects prudent use, improves medical outcomes and stewardship principles by delinking financial reward from volume of prescriptions
Is complementary to existing and planned initiatives, such as HERA and the ECDC increased competences
Provides an opportunity for the EU to lead in the development of a new form of incentive replicable in other regions

FUTURE PROOFING: ADAPTED, AGILE AND PREDICTABLE REGULATORY FRAMEWORK FOR NOVEL PRODUCTS

Novel products and innovative solutions continue to challenge the understanding of a “medicinal product” with low volume, and cutting-edge products (e.g. medicines combined with self-learning artificial intelligence) becoming a new reality. ‘Bedside’ manufacture of more individualised medicines changes the way medicines are produced. There are classification and interplay challenges with other medical products, such as medical devices and substances of human origin, or related to the combination of clinical trials with in vitro diagnostics/medical devices and medicines. In addition, certain cell-based advanced therapy medicines[8] are offered in hospital settings and are exempted from aspects of the pharmaceutical legislation. These developments offer possibilities for novel promising treatments and new ways of authorising and monitoring medicines but they are also testing the limits of the current regulatory system. They need to be addressed to unfold their potential while safeguarding the principles of high quality, safety and efficacy of medicines.

Digital transformation is affecting the discovery, development, manufacture, evidence generation, assessment, supply and use of medicines. Medicines, medical technologies and digital health are becoming increasingly integral to overarching therapeutic options. These include systems based on artificial intelligence for prevention, diagnosis, better treatment, therapeutic monitoring and data for personalised medicines and other healthcare applications.

[8] Advanced therapy medicinal products (ATMPs) are medicines for human use that are based on genes, tissues or cells. They offer ground-breaking new opportunities for the treatment of disease and injury.

Q6 How would you assess the following measures to create an adapted, agile and predictable regulatory framework for novel products?

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Maintain the current rules.	○	○	◉	○	○	○
2. Create a central mechanism in close coordination with other concerned authorities (e.g. those responsible for medical devices, substances of human origins) to provide non-binding scientific advice on whether a treatment/product should be classified as a medicine or not.	◉	○	○	○	○	○
3. Make use of the possibility for 'regulatory sandboxes'[9] in legislation to pilot certain categories of novel products/technologies. *[9] Some very innovative solutions fail to see the light of day because of regulations which might be outdated or poorly adapted for fast evolving technologies. One way to address this is through regulatory sandboxes. This enables innovative solutions not already foreseen in regulations or guidelines to be live-tested with supervisors and regulators, provided that the appropriate conditions are in place, for example to ensure equal treatment. Regulatory sandboxes provide up-to-date information to regulators and supervisors on, and experience with, new technology, while enabling policy experimentation. See COM(2020) 103 final.*	◉	○	○	○	○	○
4. Create adaptive regulatory frameworks (e.g. adapted requirements for authorisation and monitoring with possibility to adjust easily to scientific progress) for certain novel types of medicines or low volume products (hospital preparations) in coherence with other legal frameworks (e.g. medical devices and substances of human origin[10]) and respecting the principles of quality, safety and efficacy. *[10] Substances that are donated by humans such as blood, plasma, cells, gametes, tissues and organs and are applied as therapy. Some substances of human origin can also become starting materials to manufacture medicines.*	◉	○	○	○	○	○

5. Introduce an EU-wide centrally coordinated process for early dialogue and more coordination among clinical trial, marketing authorisation, health technology assessment bodies, pricing and reimbursement authorities and payers for integrated medicines development and post-authorisation monitoring.	○	○	○	○	◉	○
6. Other (please specify)	◉	○	○	○	○	○

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

High quality assessments based on best expertise, improved efficiency, international convergence and revision of process from opinion to MA decision are needed. This should be complemented with enhanced expedited regulatory pathways (expanded eligibility, iterative and agile scientific advice, dynamic review) and use of the sandbox concept. A new legal category for combination products to be regulated as medicines would extend EMA remit, streamlining and accelerating decision-making and enabling full potential of precision medicine. It should be underpinned by a digitalisation and telematics strategy. Regulatory processes should be focused on the assessment of efficacy, safety and quality. Involvement of payers in regulatory/HTA consultations could therefore detract their scientific focus.

Q7. Do you think that certain definitions and the scope of the legislation need to be updated to reflect scientific and technological developments in the sector (e.g. personalised medicines, bedside manufacturing, artificial intelligence) and if so what would you propose to change?

1000 character(s) maximum

Europe must put in place a framework to accommodate tomorrow's innovation, with the regulatory flexibility to adapt as and when the technology does. Hard law measures are not always the most effective and meaningful due to their rigidity, increased bureaucracy and lack of adaptability. A soft law instrument is in many cases more suitable to accommodate different types of innovation, accompanied with the need for some hard, prescriptive regulatory measures due to the high degree of risk associated with many novel health technologies. See the document attached to this submission for additional information on medicinal product used with a medical device, or a device part, ATMPs, AI.
EFPIA recommends definitions for criteria of good off-label and hospital exemption use to ensure safe drug therapy if a licensed medicine does not meet needs of an individual patient, while assuring public health remains a priority and is not undermined by economic interests.

REWARDS AND OBLIGATIONS RELATED TO IMPROVED ACCESS TO MEDICINES

Some medicines and therapies do not always reach patients in all EU countries, so patients in the EU still have different levels of access to medicines, depending on where they live. Even if a medicine received an EU-wide authorisation, companies are currently not obliged to market it in all EU countries. A company may decide not to market its medicines in, or decide to withdraw them from, one or more countries. This can be due to various factors, such as national pricing and reimbursement policies, size of the population and level of wealth, the organisation of health systems and national administrative procedures. Smaller markets in particular face challenges for availability and supplies of medicines.

Q8 How would you assess the following measures to improve patient access to medicines across the EU?

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Maintain the current rules which provide no obligation to market medicines in all EU countries.	◉	○	○	○	○	○
2. Require companies to notify their market launch intentions to regulators at the time of the authorisation of the medicine.	○	○	○	◉	○	○
3. Introduce incentives for swift market launch across the EU.	○	○	○	◉	○	○
4. Allow early introduction of generics in case of delayed market launch of medicines across the EU, while respecting intellectual property rights.	○	○	○	◉	○	○
5. Require companies to place – within a certain period after authorisation – a medicine on the market of the majority of Member States, that includes small markets.	○	○	○	◉	○	○
6. Require companies withdrawing a medicine from the market to offer another company to taker over the medicine.	○	○	○	◉	○	○
7. Introduce rules on electronic product information to replace the paper package leaflet.	◉	○	○	○	○	○
8. Introduce harmonised rules for multi-country packages of medicines.	◉	○	○	○	○	○
9. Other (please specify).	◉	○	○	○	○	○

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

The causes of unavailability and delay of centrally approved products are multifactorial, mostly independent of industry will: regulatory requirements, differences in medical practices, speed of pricing and reimbursement negotiations, ability to achieve an adequate price for payers and industry, health expenditure levels, company's local resources. In most countries, the inclusion of the product on the reimbursement list will determine availability and access. We caution against using regulatory tools designed for medicines authorisation to address availability issues that are within the remit of Member States. Medicines availability, access and affordability require an evidence-based multistakeholders structured dialogue. Generics early introduction cannot be done in respect of IP rights.

ENHANCE THE COMPETITIVE FUNCTIONING OF THE MARKET TO ENSURE AFFORDABLE MEDICINES

The affordability of medicines has implications for both public and household finances. It poses a growing challenge to pay for medicines in the majority of Member States. Often, innovative medicines have higher prices, while there are growing concerns among stakeholders about the real-life effectiveness of some medicines and related overall costs. This puts the budgetary sustainability of health systems at risk, and reduces the possibilities for patients to have access to these medicines. Generics and biosimilars[11] of medicines which no longer benefit from intellectual property protection (off-patent medicines) may provide accessible and affordable treatments. They also increase the availability of alternative treatment options for patients. They may also increase competition between available medicines. However, experience shows that there are still barriers for medicines entering the EU market, including for generics or biosimilars.

[11] "Generics" are copies of medicines based on simple or chemical molecules; "biosimilars" are copies of medicines based on biological molecules.

Q9 In your view, to what extent would the following measures support access to affordable medicines?

	To a great extent	To a certain extent	No change	Very little	Not at all	Don't know
1. Maintain the current rules.	○	◉	○	○	○	○
2. Stimulate earlier market entry through a broader possibility to authorise generics /biosimilars despite ongoing patent protection ('Bolar exemption')[12]. *[12] The Bolar exemption allows companies to conduct research on patent protected medicines under the condition that it is with a view to apply for a marketing authorisation for a generic.*	○	○	◉	○	○	○

3. Create a specific (regulatory) incentive for a limited number of biosimilars that come to the market first.	○	○	◉	○	○	○
4. Introduce an EU-wide scientific recommendation on interchangeability for specific biosimilars.	○	○	○	◉	○	○
5. Introduce other, non-legislative measures, such as joint procurement to reinforce competition while addressing security of supply and environmental challenges.	○	○	○	◉	○	○
6. Other (please specify).	◉	○	○	○	○	○

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

Strong incentives must be in place for continuous and sustained investment in innovative biologics. Health care sustainability should be viewed from a holistic perspective recognising the long-term societal benefits and value creation that access to medicines provides for health systems. While competition can play an important role for patient access, it needs to create a level playing field while recognising differences between generics and biosimilars, such as increased development costs, higher disease complexity, and more targeted treatments for fewer patient populations. Fair and transparent tendering procedures should focus on multiple criteria and not only on cost, allowing for a wide variety of products from multiple suppliers and therefore limiting shortages.

REPURPOSING OF MEDICINES

Repurposing is the process of identifying a new use for an established medicine in a disease or condition other than that it is currently authorised for. Repurposing of older (off-patent) medicines constitutes an emerging and dynamic field of medicines development, often led by academic units and medical research charities, with the potential for faster development times and reduced costs as well as lower risks for companies. This is because repurposing commonly starts with substances that have already been tested and many have demonstrated an acceptable level of safety and tolerability. The objective is to identify the opportunities and address any regulatory burdens to facilitate repurposing of off-patent, affordable medicines.

Q10 What measures could stimulate the repurposing of off-patent medicines and provide additional uses of the medicine against new diseases and medical conditions? Please justify your answers.

1000 character(s) maximum

While there is no legal barrier preventing non-profit research or academic organisations from applying for marketing authorisation for a medicine in a new indication, significant practical hurdles exist: high costs, administrative burden, lack of infrastructure, expertise, and resources to comply with necessary requirements. Positive incentives are also lacking. EFPIA has been part since its initiation of a multi-

stakeholder initiative on repurposing framework for off-patent products, aiming to make the regulatory pathway easier for industry, academia and regulators. Nevertheless, currently, there are rather disincentives to truly regard repurposing as a viable way to address UMN. Economic off-label use, cross-label prescribing and P&R challenges should all be tackled. The Commission should look at the merits of incentives and how they could support repurposing, e.g. indication-based pricing and reimbursement models that could make repurposing of off-patent products more attractive.

SECURITY OF SUPPLY OF MEDICINES

Shortages of medicines and the vulnerabilities in the pharmaceutical supply chain continue to be concerns in the EU. Shortages of medicines can have serious impacts on patient care. Under the current pharmaceutical legislation, pharmaceutical companies and wholesalers must, within the limits of their responsibilities, ensure a continued supply of medicines once they are placed on the market in the EU. Companies must also notify national authorities at least two months before an expected shortage or planned market withdrawal.

Q11 What is your view on the following measures to ensure security of supply of medicines in the EU?

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Maintain the current rules.	○	○	○	◉	○	○
2. Earlier reporting of shortages and market withdrawals to national authorities in a common format.	○	◉	○	○	○	○
3. Companies to have shortage prevention plans.	◉	○	○	○	○	○
4. Companies to have safety stocks.	○	○	○	◉	○	○
5. Monitoring of supply and demand at national level.	◉	○	○	○	○	○
6. Introduce a shortage monitoring system at EU level.	◉	○	○	○	○	○
7. Require companies to diversify their supply chains, in particular the number of key suppliers of medicines and components.	○	○	○	○	◉	○
8. Companies to provide more information to regulators on their supply chain.	○	○	◉	○	○	○
9. Introduce penalties for non-compliance by companies with proposed new obligations.	○	○	○	○	◉	○
10. EU coordination to help identify areas where consolidation in the supply chain has reduced the number of suppliers.	○	○	◉	○	○	○
11. Other (please specify)	◉	○	○	○	○	○

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

Some of the above measures are likely to have no or even a counterproductive effect if applied to all categories of medicines, e.g. the diversification of supply chains could affect availability of low-volume /innovative products. Shortage prevention plans provide a constructive solution, if applied to selected categories of products. "One-size-fits-all" will fail. Further granularity is required, and generally a risk-based approach should prevail, i.e. resources/efforts invested should be commensurate of the risk. It is fundamental to set up an efficient European monitoring of stocks (based on EMVS data), demand and shortages, based on a common set of data or format, based on a common definition of a shortage (based on patient needs and a defined timeline).

QUALITY AND MANUFACTURING

Medicines manufactured for the EU market must comply with the principles and guidelines of good manufacturing practice (GMP). GMP describes the minimum standard that a medicines manufacturer must meet in their production processes. GMP requires that medicines are of consistent high quality, are appropriate for their intended use and meet the requirements of the marketing authorisation or clinical trial authorisation.

Q12 What is your opinion of the following measures to ensure manufacturing and distribution of high quality products?

	Very adequate	Adequate	Neutral	Less adequate	Not adequate	Don't know
1. Maintain the current rules.	◉	○	○	○	○	○
2. Strengthen manufacturing and oversight rules.	○	○	◉	○	○	○
3. Adapt manufacturing rules to reflect new manufacturing methods.	◉	○	○	○	○	○
4. Include selected environmental requirements for manufacturing of medicines in line with the one health approach on antimicrobial resistance[13]. *[13] The one-health approach is a holistic and multi-sectorial approach to addressing antimicrobial resistance since antimicrobials used to treat infectious diseases in*	○	○	○	○	◉	○

animals may be the same or be similar to those used in humans.						
5. Increase Member State cooperation and surveillance of the supply chain in the EU and third countries.	○	○	◉	○	○	○
6. Strengthen and clarify responsibilities of business operators over the entire supply chain on sharing information on quality, safety and efficacy.	○	◉	○	○	○	○
7. Other (please specify).	◉	○	○	○	○	○

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

Core legislation adequately covers CMC/manufacturing. The principles of GMP and GDP are independent of a specific product category. Environmental requirements are already established, maintained and enforced by DG ENV regulations. To make the EU framework future-proof, increase the agility of supply chains and accelerate innovation, we need to:
Change the Variation Regulation to support global harmonisation, post-approval changes, manufacturing and digitalisation innovation
Implement flexibilities from COVID19, align regulatory practice, and enhance the use of platform knowledge, MRAs, PIC/S
Create regulatory sandboxes to test new innovative concepts
Ensure that other policies do not create vulnerabilities in manufacturing and supply
Further support global supply chains

ENVIRONMENTAL CHALLENGES

While access to pharmaceuticals is a priority, it is also important that the environmental impacts of those pharmaceuticals are as low as possible. The environmental risk assessments (ERAs) is currently not taken into account in the overall benefit/risk analysis which influences the delivery of a marketing authorisation (MA) of a medicine. ERA can influence risk management measures. Yet, ERA results are not decisive in the MA process.

Q13 How would you assess the following measures to ensure that the environmental challenges emerging from human medicines are addressed?

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Maintain the current rules.	○	◉	○	○	○	○
2. Strengthen the environmental risk assessment during authorisation of a medicine, including risk mitigation measures, where appropriate.	○	○	◉	○	○	○
3. Harmonize environmental risk assessment by national regulators, including risk mitigation measures.	◉	○	○	○	○	○
4. Increase information to the health care professionals and the general public about the assessment of environmental risks of medicines.	○	○	◉	○	○	○
5. Allow companies to use existing data about environmental risks for authorisations of a new medicine to avoid duplicating tests.	◉	○	○	○	○	○
6. Other (please specify).	◉	○	○	○	○	○

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

The current EU ERA guidance provides a good scientific foundation to maintain consistency that will allow comparisons and benchmarks to be drawn over time. Most APIs pose low or insignificant risk and any risk refinement should precede risk mitigation or labelling measures. Providing environmental information to non-experts (e.g. HCPs) should be carefully considered, ERAs are conservative and iterative and one is not necessarily comparable to another. Patient safety comes first. Transparency and accessibility of data are critical to manage risk perceptions, minimise conflicting ERAs and prevent duplication of testing (reducing animal use). Industry proposes an extended ERA to help to prioritise ERAs and refinement efforts on the APIs that pose the greatest potential environmental risk.

Q14 Is there anything else you would like to add that has not been covered in this consultation?

900 character(s) maximum

A recent study commissioned by EFPIA found out that in 2020 alone, around 5,000 clinical trials were launched across disease areas for new treatments for unmet health needs, despite the disruption caused by COVID-19. The volume of trials has even increased over the past five years. 40% of these trials are on substances targeting rare diseases, while ground-breaking cell and gene therapies continue to grow in importance. Our capacity to continue to develop and deploy the results of these research efforts requires fresh thinking about Europe's research ecosystem and healthcare infrastructure, in line with the Pharmaceutical Strategy objective of supporting competitiveness, innovation and sustainability of the EU's pharmaceutical industry. More granularity on EFPIA position on the main topics described in the open public consultation can be found in the document accompanying our submission.

Q15 In case you would like to share a document that substantiates your replies, please upload it below (optional).

Only files of the type pdf,txt,doc,docx,odt,rtf are allowed

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